As confidentially submitted to the Securities and Exchange Commission on November 24, 2021. This Amendment No. 2 to the draft registration statement has not been publicly filed with the Securities and Exchange Commission and all information herein remains confidential.

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

Amylyx Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	2834	46-4600503
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

43 Thorndike St.
Cambridge, Massachusetts 02141
(617) 682-0917
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Joshua B. Cohen, Co-Chief Executive Officer
Justin B. Klee, Co-Chief Executive Officer
Amylyx Pharmaceuticals, Inc.
43 Thorndike St.
Cambridge, Massachusetts 02141
(617) 682-0917
(Name, address, including zip code, and telephone number, including area code, of agent for service)

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Approximate date of commencement of proposed sale to the public: As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933 check the following box. ☐

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act. ☐

CALCULATION OF REGISTRATION FEE

Class of Securities To Be Registered	Proposed Maximum Aggregate Offering Price(1)	Amount of Registration Fee(2)
Common Stock, $0.0001 par value per share	$	$

(1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.
(2) Calculated pursuant to Rule 457(o) under the Securities Act of 1933, as amended, based on an estimate of the proposed maximum aggregate offering price. Includes the offering price of additional shares of common stock that the underwriters have the option to purchase.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any state where the offer or sale is not permitted.

Subject To Completion. Dated , 2021.

Shares

Common Stock

This is an initial public offering of common stock by Amylyx Pharmaceuticals, Inc.

We are offering shares of our common stock.

Prior to this offering, there has been no public market for the common stock. It is currently estimated that the initial public offering price per share will be between $ and $. We have applied to list our common stock on the Nasdaq Global Market under the symbol "AMLX."

We are an "emerging growth company" as defined under U.S. federal securities laws and, as such, will be subject to reduced public company reporting requirements for this prospectus and future filings.

Investing in our common stock involves risks. See "Risk Factors" beginning on page 13 to read about factors you should consider before buying shares of our common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities nor passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	$	$
Underwriting discounts(1)	$	$
Proceeds, before expenses, to Amylyx Pharmaceuticals, Inc.	$	$

(1) See the section titled "Underwriting" for a description of the compensation payable to the underwriters.

To the extent that the underwriters sell more than shares of common stock, the underwriters have the option to purchase up to an additional shares from us at the initial price to the public less the underwriting discount.

The underwriters expect to deliver the shares against payment in New York, New York on , 2021.

Goldman Sachs & Co. LLC **SVB Leerink** **Evercore ISI**

H.C. Wainwright & Co.

Prospectus dated , 2021

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Neither we nor the underwriters have authorized anyone to provide you with any information or to make any representations other than those contained in this prospectus, any amendment or supplement to this prospectus or in any free writing prospectus we may authorize to be delivered or made available to you. Neither we nor the underwriters take responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date on the front cover of this prospectus, regardless of the time of delivery of this prospectus or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: We have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside the United States.

In this prospectus, unless otherwise stated or the context otherwise requires, references to "Amylyx," "the Company," "we," "us," "our" and similar references refer to Amylyx Pharmaceuticals, Inc. Amylyx and other trademarks or service marks of Amylyx appearing in this prospectus are the property of Amylyx. The other trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

SUMMARY

This summary highlights, and is qualified in its entirety by, the more detailed information and financial statements included elsewhere in this prospectus. This summary does not contain all of the information that may be important to you. You should carefully consider, among other things, the matters discussed in "Risk Factors," "Special Note Regarding Forward-Looking Statements and Industry Data," "Management's Discussion and Analysis of Financial Condition and Results of Operations", and our consolidated financial statements and the accompanying notes, in each case included elsewhere in this prospectus.

Overview

Our mission is to develop therapies that change the treatment paradigm for amyotrophic lateral sclerosis, or ALS, and a broad range of neurodegenerative diseases by keeping neurons alive. Unlike most other cells in the body that regularly die and are replaced as part of healthy function, mature neurons are normally resistant to cell death and generally cannot regenerate. We are pursuing commercialization of our product candidate, AMX0035, which we believe is the first drug candidate to show both a functional and survival benefit in a large-scale clinical trial of patients with ALS. We submitted a New Drug Submission, or NDS, in Canada in the second quarter of 2021 for AMX0035 for the treatment of ALS and a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, in the fourth quarter of 2021. We also intend to submit a Marketing Authorization Application, or MAA, in Europe in the first quarter of 2022. The results of our Phase 2 clinical trial of AMX0035, known as the CENTAUR trial, were published in September 2020 in the *New England Journal of Medicine* and in October 2020 in the *Journal of Muscle and Nerve* and demonstrated functional and survival benefits for ALS patients. We believe AMX0035 has the potential to be a foundational therapy, meaning that it could be used alone or in conjunction with other therapies to change the treatment paradigm across a broad range of neurodegenerative diseases.

AMX0035 is a dual UPR-Bax apoptosis inhibitor composed of sodium phenylbutyrate, or PB, and TURSO (also known as tauroursodeoxycholic acid, or TUDCA). Through the resolution of the unfolded protein response, or UPR, and by inhibiting translocation of the Bcl-2 Associated X-protein, or Bax, to the outer mitochondrial membrane, we have shown in multiple models that AMX0035 can keep neurons alive under a variety of different conditions and stresses, including in *in vitro* models of neurodegeneration, endoplasmic reticulum stress, mitochondrial dysfunction, oxidative stress and disease-specific models of a variety of other conditions, as well as *in vivo* models of ALS, Alzheimer's Disease, or AD, and multiple sclerosis. We are pursuing ALS as our first indication as it is a disease of rapid and profound neurodegeneration.

We are actively pursuing regulatory approvals of AMX0035 for the treatment of ALS in Canada, the United States and Europe. We have recently initiated a Phase 3 clinical trial of AMX0035 for the treatment of ALS, known as the PHOENIX trial, at clinical trial sites in the United States and Europe. Based on dialogue with the FDA prior to our NDA submission, including at a pre-NDA meeting recommended by the FDA and subsequent discussions, we believe that data from the PHOENIX trial will not be required for the FDA to make a determination on the approval of AMX0035 for the treatment of ALS, although there can be no assurance that the FDA will not require further data before making a determination. The PHOENIX trial is designed to provide further data supporting the safety and efficacy of AMX0035 for the treatment of ALS to further support our global regulatory efforts.

In addition, we are developing AMX0035 for other neurodegenerative diseases by leveraging our deep knowledge of and relationships in the neurodegenerative space. We believe the approach of a

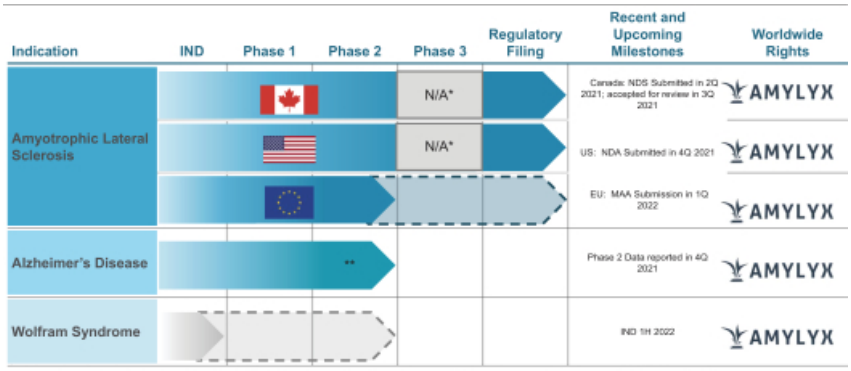
dual UPR-Bax apoptosis inhibitor designed to help keep neurons alive could be clinically meaningful for the treatment of other neurodegenerative disease indications in addition to ALS. Many common and rare neurodegenerative diseases are characterized by substantial neuronal cellular loss, including AD, and Wolfram syndrome, as well as Parkinson's Disease, Huntington's Disease, Progressive Supranuclear Palsy, Multi-System Atrophy, and others. We conducted a Phase 2 clinical trial in AD, known as the PEGASUS trial, to obtain safety data along with initial efficacy and biomarker data which could help us prioritize additional indications to pursue with AMX0035. We believe the topline results from the PEGASUS trial, reported in November 2021, provide further biological knowledge about AMX0035 which will help inform future clinical development of AMX0035 for the treatment of AD and in other potential indications. We will continue to evaluate these data and discuss the results of the PEGASUS trial with scientific advisors as we consider potential next steps for the development of AMX0035 for the treatment of AD within our clinical development strategy. Based on preclinical evidence, we are planning to pursue a clinical trial in Wolfram syndrome. We intend to prioritize our development efforts around neurodegenerative diseases that result in substantial disability, and ultimately death, and where unmet medical needs are greatest.

Neurodegeneration represents one of today's most significant unmet medical needs. The development of therapies that preserve neuron health has historically presented unique challenges, including an imperfect understanding of underlying biology and a lack of translation of activity observed in preclinical studies to results in clinical trials. Currently approved therapies for many neurodegenerative diseases are generally only symptom modifying and have demonstrated limited efficacy. There remains an urgent need for novel approaches to address most neurodegenerative diseases, especially for progressive and severe conditions such as ALS. Since our founding in 2013, our goal has been to improve the quality of, and extend, life for patients suffering from neurodegenerative diseases. One of our key strategies towards achieving this goal has been to form direct relationships with patients, their families, advocacy groups, and healthcare professionals to bring much needed innovation to patients. These relationships are a cornerstone of our culture and corporate strategy.

Pipeline Overview

AMX0035 is a proprietary oral fixed-dose combination of two small molecules: PB, which is a small molecular chaperone that reduces the UPR, preventing cell death resulting from the UPR, and TURSO, which is a Bax inhibitor that reduces cell death through apoptosis. While the PB and TURSO molecules individually are not proprietary to us, we own patents and patent applications covering AMX0035, including the fixed-dose combination of AMX0035 itself. We believe that our proprietary combination of these two mechanisms of action will allow us to target abnormal cell death to better prevent neurodegeneration than treatment with either mechanism of action alone.

Our current pipeline, including the stage of development of AMX0035 in our target indications, is represented in the table below.



Clinical Development of AMX0035 for ALS and Other Indications

We published detailed results from the CENTAUR trial in September 2020 in the *New England Journal of Medicine and in October 2020 in the Journal of Muscle and Nerve. The CENTAUR trial was a randomized, double-blind, placebo-controlled trial conducted at 25 centers of the Northeast ALS Consortium, or NEALS, and evaluated 137 adult patients with ALS. Participants that completed the randomized period were given the option to enroll in the open-label extension, or OLE, trial in which all participants received AMX0035 for up to 35 months. We designed our Phase 2 CENTAUR trial with input from leading ALS experts from NEALS to detect a significant difference between AMX0035 and placebo while providing the option for participants to continue with available approved therapies for the duration of the trial.*

The primary efficacy outcome measure for the CENTAUR trial was the rate of decline in the Revised ALS Functional Rating Scale, or ALSFRS-R total score. The ALSFRS-R scale is the most widely used ALS rating scale in ALS clinical practice and in ALS clinical trials. The CENTAUR trial met its primary endpoint with a statistically significant slowing of functional decline among participants randomized to AMX0035 (n=87) compared to placebo (n=48) (p-value of 0.03). These results showed that patients receiving AMX0035 scored an average of 2.32 points higher on the ALSFRS-R as compared to patients receiving placebo after 24 weeks, a difference of 25%. In a survey of ALS clinicians and researchers conducted and sponsored by NEALS, with the objective of determining what percentage reduction in ALSFRS-R would be considered clinically meaningful, a difference of greater than or equal to 20% in ALSFRS-R total score was considered clinically meaningful by a majority of clinicians and researchers surveyed.

Overall survival, or OS, was analyzed for all subjects randomized in the CENTAUR trial (intention to treat, or ITT, analysis) and compared patients originally randomized to AMX0035 (n=89) with those randomized to placebo (n=48). The risk of death was 44% lower among those originally randomized to AMX0035 compared with those originally randomized to placebo (hazard ratio, or HR, of 0.56; a 95% confidence interval, or CI, ranging from 0.34 to 0.92; and a p-value of 0.023). Median survival duration was 25.0 months (95% CI of 19.0 to 33.6 months) in the group previously randomized to AMX0035 and

18.5 months (95% CI of 13.5 to 23.2 months) in the group previously randomized to placebo. Participants originally randomized to AMX0035 received a median 6.9 months greater AMX0035 exposure than those originally randomized to placebo.

AMX0035 was generally well-tolerated with an adverse event rate similar to placebo. Adverse events, or AEs, were reported in 97% (86 out of 89) of participants receiving AMX0035 and 96% (46 out of 48) of participants receiving placebo, with the nature of the AEs being substantially similar in both groups. We believe AMX0035 is the first drug candidate in ALS to demonstrate a statistically significant benefit both in function as measured by a prespecified mean rate change in ALSFRS-R and in a longer-term analysis of OS.

We submitted an NDS for AMX0035 for the treatment of ALS to Health Canada in the second quarter of 2021, which was accepted for review in the third quarter of 2021, as well as an NDA for AMX0035 for the treatment of ALS to the FDA in the fourth quarter of 2021. We anticipate that the next steps in the clinical development plan of AMX0035 in ALS will be as follows:

- We intend to submit an MAA for approval of AMX0035 for the treatment of ALS to the European Medicines Agency's Committee for Medicinal Products for Human Use, or CHMP, in the first quarter of 2022.
- We recently initiated our global 48-week randomized, double-blind, placebo-controlled Phase 3 PHOENIX trial in the fourth quarter of 2021. We expect to recruit approximately 600 patients from 55 European and U.S. sites. The primary endpoint of our PHOENIX trial is a composite measure of survival and ALSFRS-R total score progression over 48 weeks. Based on dialogue with the FDA prior to our NDA submission, including at a pre-NDA meeting recommended by the FDA and subsequent discussions, we believe that data from the PHOENIX trial will not be required for the FDA to make a determination on the approval of AMX0035 for the treatment of ALS, although there can be no assurance that the FDA will not require further data before making a determination. This trial is designed to provide further data supporting the safety and efficacy of AMX0035 for the treatment of ALS to further support our global regulatory efforts.

Any regulatory approvals we may receive may be limited or subject to restrictions or post-approval commitments.

We are also developing AMX0035 for the treatment of AD. We designed our multicenter, randomized, double-blind, placebo-controlled Phase 2 PEGASUS trial with AD experts to evaluate the safety, tolerability and activity of AMX0035 in patients with late mild cognitive impairment or early-to-moderate dementia. We announced the completion of our PEGASUS trial in November 2020 and announced topline results in the fourth quarter of 2021.

We believe AMX0035 also has the potential to provide benefit in a number of additional neurodegenerative indications. We are prioritizing these conditions on an indication-by-indication basis, based on the following criteria: the strength of the data supporting AMX0035's potential benefit; the urgency of the unmet need; the practicality of conducting clinical studies in these conditions; the efficiency of clinical development activities; and the commercial potential.

Our Strategy

Our mission is to change the treatment paradigm for neurodegenerative diseases. Key elements of our strategy to achieve this mission include:

- *Obtaining regulatory approval of AMX0035 for ALS in Canada, the United States and Europe.*

- *Effectively and efficiently commercializing AMX0035 for ALS in key territories, if approved.*
- *Maximizing the therapeutic potential of AMX0035 by expanding into additional neurodegenerative diseases.*
- *Continuing to cultivate a network of patient advocacy groups, key opinion leaders, research institutions, and healthcare professionals to inform our patient-centric approach.*
- *Deploying a strategic approach to design, acquire and develop new therapies.*

Our Company and Team

Amylyx was founded with the ambitious goal of improving the quality and length of life for patients suffering from neurodegenerative diseases. From a dorm room at Brown University in 2013, our Co-CEOs and Co-Founders, Josh Cohen and Justin Klee, set out to determine why neurons die, and have ever since been working to develop AMX0035, which we believe is the first drug candidate to show a clear effect on function and survival in ALS, and other novel therapies. To help realize our goal, we have assembled a team with deep scientific, clinical, business and leadership experience, bolstered by expertise in biotechnology. Our Chief Financial Officer, James Frates, brings over 20 years of experience as the Chief Financial Officer of Alkermes. Our Chief Commercial Officer, Margaret Olinger, brings three decades of expertise in commercial launches and operations, most recently at Alexion. Our Global Head of Supply, Tom Holmes, brings more than 25 years of leadership experience at Biogen in supply chain, biopharmaceutical manufacturing and program management. Our Global Head of Clinical Research & Development and Chief Medical Officer, Patrick D. Yeramian, brings over 30 years of medical and pharmaceutical industry experience. Our Head of Regulatory Affairs, Tammy Sarnelli, brings more than 30 years of experience from Biogen and other companies in early and late-stage neurology and rare disease development. Our Global Head of Human Resources, Debra Canner, brings over 20 years of experience, having served as the Chief Human Resources Officer at Akamai and as part of Genzyme. This team brings a diverse set of skills uniquely suited to drive successful commercialization of AMX0035 in ALS while continuing to advance AMX0035 in other indications.

Risks Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus immediately following this prospectus summary. These risks include the following:

- **Risks Related to Our Financial Position and Need for Capital**
  - *We are a clinical-stage biopharmaceutical company and we have incurred significant losses since our inception. We anticipate that we will continue to incur significant losses for the foreseeable future.*
  - *We have never generated revenue from product sales and may never be profitable.*
  - *We have a limited operating history and only one product candidate, AMX0035, which is in preclinical studies and clinical trials and has no commercial sales, which may make it difficult to evaluate the prospects for our future viability.*
- **Risks Related to the Discovery and Development of Our Current or Future Product Candidates**
  - *We currently depend on the success of AMX0035, which is our only product candidate. If we are unable to obtain regulatory approval for, and successfully commercialize, AMX0035, or experience significant delays in doing so, our business may be materially harmed.*
  - *The denial of regulatory approval for AMX0035 in any jurisdiction could mean that we need to delay or even cease operations, and a delay in obtaining such approval could delay commercialization of AMX0035 and adversely impact our ability to generate revenue, our business and our results of operations.*
  - *We have never commercialized a product candidate and may experience delays or unexpected difficulties in obtaining regulatory approval for AMX0035 for our initial or potential additional indications.*
  - *AMX0035 is a fixed-dose combination drug product and certain regulatory authorities, including the FDA, require a demonstration that each component makes a contribution to the claimed effects in addition to demonstrating that the combination is safe and effective for the intended population.*
  - *We have concentrated our research and development efforts on the treatment of neurodegenerative and central nervous system, or CNS, disorders, a field that has seen very limited success in product development.*
  - *The regulatory approval processes of the FDA, Health Canada, the EMA and other comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for AMX0035 or any future product candidates, our business will be substantially harmed.*
  - *Competitive products may reduce or eliminate the commercial opportunity for AMX0035 for our current or future indications. If our competitors develop technologies or product candidates more rapidly than we do, or their technologies or product candidates are more effective or safer than ours, our ability to develop and successfully commercialize AMX0035 may be adversely affected.*
- **Risks Related to Our Dependence on Third Parties**
  - *We may in the future enter into collaborations with third parties for the development and commercialization of AMX0035 or any future product candidates, and our prospects with respect to those product candidates will depend in significant part on the success of those collaborations. For example, Humanitas Mirasole SpA, an entity we have no relationship with, is conducting a Phase 3 clinical trial in* the EU to assess the safety and efficacy of TURSO in

patients with ALS which may lead to additional findings as to the safety profile of TURSO. If their Phase 3 clinical trial is successful and TURSO is approved by the FDA or any other regulatory agency, TURSO may become a commercialized product competitive with AMX0035, unless our intellectual property protections and any regulatory exclusivities we possess or may possess in the future prevent such commercialization.

- *We rely on third parties to assist in conducting our clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or commercialize AMX0035 or any future product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.*
- *Our use of third parties to manufacture AMX0035 may increase the risk that we will not have sufficient quantities of AMX0035, products, or necessary quantities of such materials on time or at an acceptable cost.*

- **Risks Related to Commercialization of AMX0035 or Future Product Candidates**
  - *We currently have limited sales and marketing capabilities. If we are unable to establish effective sales and marketing capabilities or enter into agreements with third parties to market and sell AMX0035 and any future product candidates that may be approved, we may not be successful in commercializing AMX0035 and any future product candidates if and when approved, and we may be unable to generate any product revenue.*
  - *Even if AMX0035 or any future product candidate of ours receives regulatory approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.*
  - *Healthcare insurance coverage and reimbursement may be limited or unavailable for AMX0035 and any future product candidates, if approved, which could make it difficult for us to sell any product candidates or therapies profitably.*
- **Risks Related to Our Intellectual Property**
  - *Our commercial success depends on our ability to protect our intellectual property and proprietary technology.*
- **Risks Related to Our Business Operations, Employee Matters and Managing Growth**
  - *A pandemic, epidemic, or outbreak of an infectious disease, such as the COVID-19 pandemic, may materially and adversely affect our business, including our preclinical studies, clinical trials, third parties on whom we rely, our supply chain, our ability to raise capital, our ability to conduct regular business and our financial results.*
  - *We depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.*
  - *We expect to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.*
- **Risks Related to Our Common Stock and this Offering**
  - *If you purchase shares of our common stock in this offering, you will incur immediate and substantial dilution.*
  - *We do not know whether an active, liquid and orderly trading market will develop for our common stock or what the market price of our common stock will be and as a result it may be difficult for you to sell your shares of our common stock.*
  - *If we fail to remediate our material weaknesses over financial reporting controls and to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations, meet our reporting obligations or prevent fraud, and investor confidence in our company and the market price of our common stock may be materially and adversely affected.*

Our Corporate Information

We were incorporated under the laws of the State of Delaware on January 10, 2014 under the name Amylyx Pharmaceuticals, Inc. We have two wholly owned subsidiaries, Amylyx Pharmaceuticals Canada Inc., which was formed in 2020, and Amylyx Pharmaceuticals EMEA B.V., which was formed in 2021. Our executive offices are located at 43 Thorndike Street, Cambridge, Massachusetts 02141, and our telephone number is (617) 682-0917. Our website address is www.amylyx.com. The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

Implications of Being an Emerging Growth Company and a Smaller Reporting Company

As a company with less than $1.07 billion of revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure and other requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- only two years of audited financial statements in addition to any required unaudited interim financial statements with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- reduced disclosure about our executive compensation arrangements;
- no non-binding advisory votes on executive compensation or golden parachute arrangements; and
- exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting.

In particular, in this prospectus, we have not included all of the executive compensation-related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock. We have elected not to "opt out" of the exemption for the delayed adoption of certain accounting standards and, therefore, we will adopt new or revised accounting standards only at the time private companies adopt the new or revised accounting standard and will do so until such time that we either (i) irrevocably elect to "opt out" of such extended transition period or (ii) no longer qualify as an emerging growth company.

We are also a "smaller reporting company," meaning that the market value of our stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our stock held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our future annual reports on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

THE OFFERING

Common stock offered by us	shares
Option to purchase additional shares	We have granted the underwriters a 30-day option to purchase up to shares of our common stock at the initial public offering price, less the underwriting discounts and commissions.
Common stock to be outstanding immediately following this offering	shares (shares if the underwriters exercise their option to purchase additional shares of common stock in full).

We estimate that the net proceeds to us from this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, will be approximately $ million, or approximately $ million if the underwriters exercise their option to purchase additional shares from us in full, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus.

	We intend to use the net proceeds from this offering, together with our existing cash, cash equivalents and investments, to fund: (i) the regulatory approval process and pre-commercial launch, production of and, if approved, commercial launch activities for AMX0035 for the treatment of ALS; (ii) the completion of our ongoing Phase 3 PHOENIX clinical trial for the treatment of amyotrophic lateral sclerosis, or ALS; (iii) the development and expansion of our pipeline to address other neurodegenerative indications, and for formulations and derivatives of AMX0035; and (iv) working capital and other general corporate activities, including the continued build out of our organization. See the “Use of Proceeds” section in this prospectus for a more complete description of the intended use of proceeds from this offering.
Risk factors	You should read the “Risk Factors” section of this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.

Exchange symbol “AMLX”

The number of shares of our common stock to be outstanding after this offering is based on 6,799,157 shares of our common stock outstanding as of September 30, 2021 and 39,474,330 additional shares of our common stock issuable upon the automatic conversion of all outstanding shares of our preferred stock upon the closing of this offering, and excludes:

- 4,195,341 shares of our common stock issuable upon the exercise of stock options outstanding as of September 30, 2021, at a weighted average exercise price of $4.31 per share;
- 2,809,492 shares of our common stock available for future issuance as of September 30, 2021 under our 2015 Stock Option and Grant Plan, or the 2015 Plan, which will cease to be available for issuance at the time that our 2022 Stock Option and Incentive Plan, or the 2022 Plan, becomes effective;
- shares of our common stock that will become available for future issuance under our 2022 Stock Option and Incentive Plan, which will become effective in connection with the completion of this offering, as well as any future increases, including annual automatic evergreen increases, in the number of shares of common stock reserved for issuance thereunder in accordance with the terms of such plan; and
- shares of our common stock that will become available for future issuance under our 2022 Employee Stock Purchase Plan, which will become effective in connection with the completion of this offering.

Unless otherwise indicated, all information in this prospectus assumes:

- no exercise of the outstanding options described above;
- no exercise by the underwriters of their option to purchase up to additional shares of our common stock;
- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of shares of our common stock upon the closing of this offering;
- a -for-1 forward split of our common stock effected on ; and
- the filing and effectiveness of our amended and restated certificate of incorporation and the amendment and restatement of our bylaws prior to the closing of this offering.

SUMMARY CONSOLIDATED FINANCIAL DATA

You should read the following summary consolidated financial data together with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements, related notes and other financial information included elsewhere in this prospectus. The summary consolidated financial data in this section are not intended to replace the consolidated financial statements and are qualified in their entirety by the consolidated financial statements and related notes included elsewhere in this prospectus. We have derived the consolidated statement of operations data for the years ended December 31, 2019 and 2020 from our audited consolidated financial statements appearing elsewhere in this prospectus. The consolidated statements of operations data for the nine months ended September 30, 2020 and 2021 and the consolidated balance sheet data as of September 30, 2021 have been derived from our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus and have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, necessary for a fair statement of the financial information in those statements. Our historical results are not necessarily indicative of results that may be expected in the future.

	Nine Months ended September 30,		Year Ended December 31,	
(in thousands, except per share and per share amount)	2020	2021	2019	2020
Consolidated Statements of Operations Data:				
Grant revenue	$ 300	$ 285	$ 1,426	$ 650
Operating expenses:				
Research and development	19,581	30,646	11,899	24,594
General and administrative	11,132	24,012	3,081	15,061
Total operating expenses	30,713	54,658	14,980	39,655
Loss from operations	(30,413)	(54,373)	(13,554)	(39,005)
Other income (expense), net:				
Interest income	14	6	176	14
Interest expense	(2,287)	—	(1,276)	(2,288)
Change in fair value of derivative liability	(1,270)	—	939	(1,270)
Change in fair value of convertible notes	—	(5,228)	—	—
Other (expense) income, net	268	8	(1)	269
Total other expense, net	(3,275)	(5,214)	(162)	(3,275)
Net loss	(33,688)	(59,587)	(13,716)	(42,280)
Net loss per share attributable to common stockholders—basic and diluted(1)	$ (5.55)	$ (9.20)	$ (2.33)	$ (6.96)
Weighted-average common shares used to compute net loss per share attributable to common stockholders—basic and diluted (1)	6,069,726	6,477,140	5,889,138	6,077,758

(1) See Notes 2 and 13 to our audited consolidated financial statements and Note 11 to our unaudited condensed consolidated financial statements, included elsewhere in this prospectus, for an explanation of the method used to calculate historical basic and diluted net loss per share attributable to common stockholders and the weighted-average common shares outstanding used in the computation of the per share amount.

	As of September 30, 2021		
	Actual	Pro Forma (2)	Pro Forma as Adjusted (3)
		(in thousands)	
Consolidated balance sheet data:			
Cash, cash equivalents, and short-term investments	$ 125,702	$ 125,702	
Working capital (1)	114,796	114,796	
Total assets	130,460	130,460	
Redeemable convertible preferred shares	239,351	—	
Common shares	1	5	
Additional paid-in capital	3,431	242,778	
Accumulated deficit	(127,501)	(127,501)	
Accumulated other comprehensive loss	(1)	(1)	
Total stockholders' (deficit) equity	(124,070)	115,281	

(1) We define working capital as current assets less current liabilities. See our consolidated financial statements appearing elsewhere in this prospectus for further details regarding our current assets and current liabilities.

(2) Pro forma consolidated balance sheet data give effect to the automatic conversion of all outstanding shares of redeemable convertible preferred stock into shares of common stock upon the closing of this offering.

(3) The pro forma as adjusted consolidated balance sheet data give further effect to the issuance and sale of shares of common stock in this offering at an assumed initial public offering prices of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The pro forma as adjusted information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash, cash equivalents, and short-term investments, working capital, total assets and total stockholders' deficit by $, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, an increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash, cash equivalents, and short-term investments, working capital, total assets and total stockholders' deficit by $, assuming no change in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

RISK FACTORS

Investing in our common stock involves a high degree of risk. Before you decide to invest in our common stock, you should consider carefully the risks described below, together with the other information contained in this prospectus, including our financial statements and the related notes appearing at the end of this prospectus. We believe the risks described below are the risks that are material to us as of the date of this prospectus. If any of the following risks actually occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks Related to Our Financial Position and Need for Capital

We are a clinical-stage biopharmaceutical company and we have incurred significant losses since our inception. We anticipate that we will continue to incur significant losses for the foreseeable future.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale and have not generated any revenue from product sales to date, and we will continue to incur significant research and development and other expenses related to our clinical development and ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. Since our inception, we have devoted the majority of our financial resources and efforts to research and development, including preclinical studies and our clinical trials, and preparation for commercialization. Our financial condition and operating results, including net losses, may fluctuate significantly from quarter to quarter and year to year. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance. Additionally, net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. Our net losses were $13.7 million and $42.3 million for the years ended December 31, 2019 and 2020, respectively, and $33.7 million and $59.6 million for the nine months ended September 30, 2020 and 2021, respectively. As of September 30, 2021, we had an accumulated deficit of $127.5 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for our product candidate, AMX0035, for the treatment of amyotrophic lateral sclerosis, or ALS, Alzheimer's disease, or AD, and potential additional indications, as well as for future product candidates we may develop.

We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct clinical trials for AMX0035 for the treatment of ALS, AD and potential additional indications;
- initiate and continue research, preclinical and clinical development efforts for any future product candidates;
- seek to identify additional product candidates;
- seek regulatory approvals in Canada, the United States, the European Union, or EU, and other geographies for AMX0035 for the treatment of ALS, AD and other indications that successfully complete clinical development;
- experience any delays or encounter any issues with any of the above, including but not limited to failed studies, negative or mixed clinical trial results, safety issues or other regulatory challenges, the risk of which in each case may be exacerbated by the ongoing COVID-19 pandemic;

- add operational, financial and management information systems and personnel, including personnel to support our product candidate development and help us comply with our obligations as a public company;
- hire and retain additional personnel, such as clinical, quality control, scientific, commercial and administrative personnel;
- maintain, expand and protect our intellectual property portfolio;
- establish sales, marketing, distribution, manufacturing, supply chain and other commercial infrastructure in the future to commercialize various products for which we may obtain regulatory approval;
- add equipment and physical infrastructure to support our research and development; and
- acquire or in-license other product candidates and technologies.

Our expenses could increase beyond our expectations if we are required by Health Canada, the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or the EMA, or other regulatory authorities to perform clinical trials or conduct other studies in addition to those that we currently expect, or if there are any delays in establishing appropriate manufacturing arrangements for or in completing our clinical trials or the development of AMX0035 or any future product candidates we may develop.

We have never generated revenue from product sales and may never be profitable.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue, if any, unless and until we, either alone or with a collaborator, are able to obtain regulatory approval for, and successfully commercialize, AMX0035 for our initial and potential additional indications, or any future product candidates we may develop. Successful commercialization will require achievement of many key milestones, which vary by jurisdiction and may include demonstrating safety and efficacy in clinical trials, obtaining regulatory, including marketing, approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our future collaborators, may obtain regulatory approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately and precisely predict the timing and amount of revenues, the extent of any further losses or if or when we might achieve profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our failure to become and remain profitable may depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. If we continue to suffer losses as we have in the past, investors may not receive any return on their investment and may lose their entire investment.

We have a limited operating history and only one product candidate, AMX0035, which is in preclinical and clinical trials and has no commercial sales, which may make it difficult to evaluate the prospects for our future viability.

We are a biopharmaceutical company founded in 2014, and our operations to date have been limited to organizing, staffing and financing our company, raising capital, and conducting research and development activities, including preclinical studies and clinical trials, for AMX0035. We have not yet

demonstrated an ability to generate revenues, obtain regulatory approvals, manufacture a commercial product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Accordingly, you should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in clinical development, especially clinical-stage biopharmaceutical companies such as ours. Any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We are preparing to transition from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Even if we consummate this offering, we will need substantial additional funding, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product discovery and development activities or commercialization efforts.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to continue the preclinical and clinical development of AMX0035 and any future product candidates. If we are able to gain marketing approval for AMX0035 or any future product candidates that we develop, including any indication for which we are developing or may develop AMX0035, we will require significant additional amounts of cash in order to launch and commercialize such product candidates to the extent that such launch and commercialization are not the responsibility of a future collaborator that we may contract with in the future. In addition, other unanticipated costs may arise in the course of our development efforts. Because the design and outcome of our ongoing and anticipated clinical trials is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of any product candidate we develop.

Our future capital requirements depend on many factors, including:

- the scope, progress, results and costs of researching and developing AMX0035 for the treatment of ALS, AD and potential additional indications, as well as any future product candidates we may develop;
- the timing of, and the costs involved in, obtaining marketing approvals for AMX0035 for the treatment of ALS, AD and potential additional indications, and any future product candidates we may develop and pursue;
- the number of future product candidates that we may pursue and their development requirements;
- if approved, the costs of commercialization activities for AMX0035 for any approved indications, or any other product candidate that receives regulatory approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of regulatory approval, revenue, if any, received from commercial sales of AMX0035 for any approved indications or any future product candidates;
- the extent to which we in-license or acquire rights to other products, product candidates or technologies;
- our headcount growth and associated costs as we expand our research and development, increase our office space, and establish a commercial infrastructure;

- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights, including enforcing and defending intellectual property related claims; and
- the ongoing costs of operating as a public company.

We cannot be certain that additional funding will be available on acceptable terms, or at all. As a result of the ongoing COVID-19 pandemic and actions taken to slow its spread, the global credit and financial markets have experienced extreme volatility and disruptions, including diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, and uncertainty about economic stability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly or more dilutive.

We have no committed source of additional capital and if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of AMX0035 or any future product candidates or other research and development initiatives. We may need to seek collaborators for AMX0035 and any future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to AMX0035 and any future product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves. Any of the above events could significantly harm our business, prospects, financial condition, and results of operations and cause the price of our common stock to decline.

We believe that the net proceeds from this offering, together with our existing cash, cash equivalents, and short-term investments, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least twelve months after the registration statement of which this prospectus forms a part becomes effective. Our estimate may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned.

Our history of recurring losses and anticipated expenditures raises substantial doubts about our ability to continue as a going concern. Our ability to continue as a going concern requires that we obtain sufficient funding to finance our operations.

We have incurred operating losses to date and it is possible we may never generate a profit. Our financial statements included elsewhere in this prospectus have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. If we are unable to raise sufficient capital in this offering or otherwise as and when needed, our business, financial condition and results of operations will be materially and adversely affected, and we will need to significantly modify our operational plans to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets, and the values we receive for our assets in liquidation or dissolution could be significantly lower than the values reflected in our consolidated financial statements. Our lack of cash resources and our potential inability to continue as a going concern may materially adversely affect our share price and our ability to raise new capital, enter into critical contractual relations with third parties and otherwise execute our development strategy.

Raising additional capital may cause dilution to our stockholders, including purchasers of common stock in this offering, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We expect our expenses to increase in connection with our planned operations. Unless and until we can generate a substantial amount of revenue from AMX0035 or any future product candidates, we

expect to finance our future cash needs through public or private equity offerings, royalty-based or debt financings, collaborations, licensing arrangements or other sources, or any combination of the foregoing. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans.

To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, your ownership interest may be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. In addition, securing financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of AMX0035 or any future product candidates.

If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us.

Changes in tax law could adversely affect our business and financial condition.

The rules dealing with U.S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

Risks Related to the Discovery and Development of Our Current or Future Product Candidates

We currently depend on the success of AMX0035, which is our only product candidate. If we are unable to obtain regulatory approval for, and successfully commercialize, AMX0035, or experience significant delays in doing so, our business may be materially harmed.

We currently only have one product candidate, AMX0035, and our business and future success depends entirely on our ability to develop, obtain regulatory approval for, and then successfully commercialize, AMX0035, which is currently in clinical development for patients with ALS and AD. To date we have obtained limited clinical trial data supporting AMX0035, having only completed a clinical trial of 137 patients with ALS and a clinical trial in 95 patients with AD. We intend to conduct additional clinical trials of AMX0035 in ALS and other indications in the future. This may make an investment in our company riskier than similar companies that have multiple product candidates in active development that may be able to better sustain failure of a lead product candidate.

We currently have no products approved for sale and are investing the majority of our efforts and financial resources in the development of our product candidate, AMX0035, for the treatment of ALS, AD and other diseases. Successful continued development and ultimate regulatory approval of

AMX0035 for our initial and potential additional indications is critical to the future success of our business. We will need to raise sufficient funds for, and successfully enroll and complete, our clinical development of AMX0035 for the treatment of ALS, AD and other indications. The future regulatory and commercial success of AMX0035 is subject to a number of risks, including the following:

- successful completion of preclinical studies and clinical trials;
- successful patient enrollment in clinical trials;
- successful data from our preclinical studies and clinical trials that supports an acceptable risk-benefit profile of AMX0035 or any future product candidates in the intended populations;
- satisfaction of applicable regulatory requirements, including to satisfy applicable rules governing fixed dose combination products;
- potential unforeseen safety issues or adverse side effects;
- receipt and maintenance of marketing approvals from applicable regulatory authorities, including with any expected new chemical entity and new clinical investigation data exclusivity and orphan drug market exclusivity;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for AMX0035 or any future product candidates;
- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of AMX0035 or any future product candidates;
- entry into collaborations to further the development of AMX0035 or any future product candidates;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- successfully launching commercial sales of AMX0035 or any future product candidates, if and when approved;
- acceptance of AMX0035 or any other products, if and when approved, by patients, the medical community and third-party payors;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- maintaining a continued acceptable safety profile of the products following approval;
- effectively competing with other therapies;
- ensuring that we promote and distribute our products consistent with all applicable healthcare laws; and
- enforcing and defending intellectual property rights and claims.

Many of these risks are beyond our control, including the risks related to clinical development, the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, receive regulatory approval for, or successfully commercialize AMX0035 for the indications we are developing it for, or if we experience delays as a result of any of these risks or otherwise, our business could be materially harmed.

In addition, of the large number of drugs in development in the pharmaceutical industry, only a small percentage result in the submission of a new drug submission, or NDS, to Health Canada, a new

drug application, or NDA, to the FDA or a marketing authorization application, or MAA, to the EMA and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval for AMX0035 for any indication, any such approval may be subject to limitations on the indications or uses or patient populations for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development activities, we cannot assure you that we will successfully develop or commercialize AMX0035 for any indication. If we or any of our future collaborators are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize AMX0035 for our initial or potential additional indications, we may not be able to generate sufficient revenue to continue our business. In addition, our failure to demonstrate positive results in our clinical trials in any indication for which we are developing AMX0035, or to satisfy other regulatory requirements could adversely affect our development efforts for AMX0035 in other indications.

The denial of regulatory approval for AMX0035 in any jurisdiction could mean that we need to delay or even cease operations, and a delay in obtaining such approval would delay commercialization of AMX0035 and adversely impact our ability to generate revenue, our business and our results of operations.

If we are not successful in commercializing AMX0035, or are significantly delayed in doing so, our business will be materially harmed, and we may need to curtail or cease operations. We currently have no drug products approved for sale, and we may never obtain regulatory approval to commercialize AMX0035 in any indication. The research, testing, manufacturing, labeling, approval, sale, marketing and distribution of drug products are subject to extensive regulation by Health Canada, the FDA, the EMA, and other regulatory agencies in the United States and other countries, and such regulations differ from country to country. We are not permitted to market AMX0035 until we receive approval of an NDA from the relevant regulatory authority. Health Canada, the FDA, the EMA or any other foreign regulatory agency can delay, limit or deny approval to market AMX0035 for many reasons, including:

- our inability to demonstrate to the satisfaction of Health Canada, the FDA, the EMA or any other applicable foreign regulatory agency that AMX0035 is safe and effective for the requested indication;
- our inability to gain agreement from applicable foreign regulatory authorities that AMX0035 is appropriate for approval under applicable regulatory pathways;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's disagreement with the interpretation of data from nonclinical and clinical studies and trials;
- our inability to demonstrate that the clinical and other benefits of AMX0035 outweigh any safety or other perceived risks;
- our ability to enroll an adequate number of patients in and successfully complete our ongoing global Phase 3 PHOENIX trial;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's requirement for additional nonclinical or clinical studies or trials, including studies to satisfy applicable rules governing fixed dose combination products;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's having differing requirements for the trial protocols used in our clinical trials;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's non-approval of the formulation, labeling and/or the specifications of AMX0035;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's failure to accept the manufacturing processes or third-party manufacturers with which we contract; or

- the potential for approval policies or regulations of Health Canada, the FDA, the EMA or any other applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of drugs in development, only a small percentage successfully complete Health Canada, the FDA, the EMA or other regulatory approval processes and are commercialized.

Even if we eventually complete clinical testing and receive approval of an NDS, NDA, MAA or foreign marketing authorization for AMX0035, the FDA or the applicable foreign regulatory agency may grant approval contingent on the performance of costly additional clinical trials, which may be required after approval. The FDA or the applicable foreign regulatory agency may also approve AMX0035 for a more limited indication and/or a narrower patient population than we originally request, and Health Canada, the FDA, the EMA or any other applicable foreign regulatory agency may not approve the labeling that we believe is necessary or desirable for the successful commercialization of AMX0035. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of AMX0035 and would materially adversely impact our business and prospects.

If the FDA does not accept our NDA for AMX0035 for filing, we will experience delays in obtaining approval of this candidate product and will incur additional costs in securing approval and commercialization of this product.

We submitted our NDA for AMX0035 for the treatment of ALS to FDA in the fourth quarter of 2021. Upon receipt of an NDA, the FDA conducts a preliminary review of the NDA within 60 days and must inform the sponsor by the 74th day after the FDA's receipt of the submission whether an application is sufficiently complete to permit substantive review. In pertinent part, FDA's regulations for NDAs state that an application "shall not be considered as filed until all pertinent information and data have been received" by the FDA. In the event that FDA determines that an NDA does not satisfy this standard, it will issue a Refuse to File, or RTF, determination to the applicant. Typically, an RTF for an NDA will be based on administrative incompleteness, such as clear omission of information or sections of required information; scientific incompleteness, such as omission of critical data, information or analyses needed to evaluate safety and efficacy or provide adequate directions for use; or inadequate content, presentation, or organization of information such that substantive and meaningful review is precluded. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted NDA is also subject to review before the FDA accepts it for filing. In the event that FDA decides not to file our NDA for review, we will experience delays in obtaining approval of AMX0035, if at all, and our ability to generate revenues will be materially impaired.

We have never commercialized a product and may experience delays or unexpected difficulties in obtaining regulatory approval for AMX0035 for our initial or potential additional indications.

Our company has never obtained regulatory approval for, or commercialized, a drug. It is possible that Health Canada, the FDA and the EMA may refuse to accept any or all of our submitted or planned NDSs, NDAs and MAAs for substantive review or may conclude after review of our data that our application is insufficient to obtain regulatory approval for AMX0035 or any future product candidates. For example, the FDA or other regulatory authorities may require completion of our ongoing Phase 3 PHOENIX global clinical trial prior to issuing an approval decision for our marketing applications for AMX0035. If Health Canada, the FDA, and the EMA do not approve any of our submitted or planned NDSs, NDAs or MAAs, such regulatory authorities may require that we conduct additional costly clinical, nonclinical or manufacturing validation studies before they will reconsider our applications. Depending on the extent of these or any other required studies, approval of any NDA or other application that we submit may be significantly delayed, possibly for several years, or may require us to expend more resources than we have available. Any failure or delay in obtaining regulatory approvals would prevent us from

commercializing AMX0035 for any indication or any other product candidate, generating revenues and achieving and sustaining profitability. It is also possible that additional studies, if performed and completed, may not be considered sufficient by the EMA or FDA to approve any MAA, NDA or other application that we submit. For example, Health Canada has indicated that the CENTAUR trial and the CENTAUR OLE trial are sufficient to support submission of our NDS; however, Health Canada has not reviewed our complete clinical data, to date, and therefore there is no guarantee that Health Canada will determine that the NDS we have submitted or any future NDS we submit to be sufficient for issuing a marketing approval of AMX0035 for ALS. If any of these outcomes occur, we may be forced to abandon the development of AMX0035 or any future product candidates, which would materially adversely affect our business and could potentially cause us to cease operations. We face similar risks for our applications in foreign jurisdictions. In addition, difficulties in obtaining approval of AMX0035 for the treatment of ALS, AD and the other indications for which we are developing AMX0035, it could adversely affect our efforts to seek approval from regulatory authorities for AMX0035 in other potential indications.

AMX0035 is a fixed-dose combination drug product and certain regulatory authorities, including the FDA, require a demonstration that each component makes a contribution to the claimed effects in addition to demonstrating that the combination is safe and effective for the intended population.

Under the combination rule, the FDA may not file or approve an NDA for a fixed-dose combination product unless each component of a proposed drug product is shown to make a contribution to the claimed effects and the dosage of each component (amount, frequency, duration) is safe and effective for the intended population. To satisfy these requirements, the FDA typically requires a clinical factorial study, designed to assess the effects attributable to each drug in the combination product. This is particularly true when the ingredients are directed at the same sign or symptom of the disease or condition.

The FDA has accepted a variety of approaches to satisfy the combination rule. In December 2015, the FDA proposed regulations that would allow the agency to waive the requirements of the combination rule for certain drug products under particular circumstances. The FDA has not, to date, finalized these regulations, but the FDA has stated that factorial studies may be unethical (e.g., omitting a drug known to improve survival) or impractical (there may be too many components to conduct a factorial study, meaning the trial cannot be conducted). The FDA has also stated that it may be possible to use other types of clinical and nonclinical data and mechanistic information available to demonstrate the contributions of the individual active ingredients to the effect of the combination.

We submitted existing preclinical and clinical data for sodium phenylbutyrate, or PB, and TURSO (also known as tauroursodeoxycholic acid, or TUDCA), in our NDA submission and the FDA has indicated it will assess the sufficiency of this information during our NDA review period. If FDA disagrees with our data and rationale, it may not approve our NDA for the treatment of ALS, or may require the successful completion of and data from our ongoing Phase 3 PHOENIX trial before issuing an approval decision. Even if FDA agrees with our data and rationale, there can be no guarantee that FDA will issue an approval decision with respect to our NDA submission. Additionally, we will be required to separately satisfy the fixed-dose combination rule for AMX0035 for the treatment of any other indications we pursue in advance of approval.

Similar requirements may be imposed on us by the EMA in the EU and comparable regulatory authorities in other jurisdictions where we intend to seek regulatory approval. While no similar combination rule formally exists in Canada, Health Canada may consider the contributions of each component in a combination product in connection with review of the NDSs. If the FDA, the EMA, Health Canada or other comparable foreign regulatory authorities require us to conduct one or more clinical trials, such as a factorial study, the design, duration, and scope of such clinical trials will be decided upon after further discussions with those agencies and other comparable foreign regulatory authorities. As a result, we are unable to predict with certainty the estimated timing or scope of any future clinical trials of AMX0035 we may be

required to conduct to satisfy these requirements governing fixed dose combination products in various jurisdictions. Ongoing third-party data in neurology, specifically within ALS on our products or other products, may influence regulatory decision making, including for fixed-dose combinations.

We have concentrated our research and development efforts on the treatment of neurodegenerative and central nervous system, or CNS, disorders, a field that has seen very limited success in product development.

We have focused our research and development efforts on addressing neurodegenerative and CNS disorders. Collectively, efforts by pharmaceutical companies in the field of neurodegenerative and CNS disorders have seen very limited successes in product development. The development of neurodegenerative and CNS therapies presents unique challenges, including an imperfect understanding of the biology, the presence of the blood brain barrier, or BBB, that can restrict the flow of drugs to the brain, a frequent lack of translatability of preclinical study results in subsequent clinical trials and dose selection, and the product candidate having an effect that may be too small to be detected using the outcome measures selected in clinical trials or if the outcomes measured do not reach statistical significance. There are few approved therapeutic options available for patients with ALS, AD and other neurodegenerative or CNS disorders. Our future success is highly dependent on the successful development of AMX0035 and any future product candidates for treating neurodegenerative and CNS disorders. Developing and, if approved, commercializing AMX0035 and any future product candidates for treatment of neurodegenerative and CNS disorders subjects us to a number of challenges, including ensuring that we have selected the optimal doses, executing an appropriate clinical trial to test for efficacy and obtaining regulatory approval from Health Canada, the FDA, the EMA and other comparable foreign regulatory authorities.

The regulatory approval processes of the FDA, Health Canada, the EMA and other comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for AMX0035 or any future product candidates, our business will be substantially harmed.

We, and any future collaborators, are not permitted to commercialize, market, promote or sell any product candidate in the United States, Canada, or the EU or without obtaining regulatory approval from the FDA, Health Canada, or the EMA, respectively. Regulatory authorities, in other jurisdictions may have similar requirements. The time required to obtain approval by the FDA, Health Canada, the EMA and other comparable foreign regulatory authorities is unpredictable, but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. While we have submitted an NDS to Health Canada and an NDA to the FDA, to date, we have not submitted a MAA to the EMA or any other similar drug approval submissions to comparable foreign regulatory authorities for AMX0035 or any other product candidate. We have not obtained approval of our NDS from Health Canada and there can be no assurance that we will receive such approval. We, and any future collaborators, must complete additional preclinical or nonclinical studies and clinical trials to demonstrate the safety and efficacy of AMX0035 or any future product candidates in humans before we will be able to obtain these approvals.

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of AMX0035 for our initial and potential additional indications or any future product candidates is susceptible to the risk of failure inherent at any stage of development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements, and determination by the FDA, Health Canada, the EMA or any other comparable foreign regulatory

authority that a product candidate may not continue development or is not approvable. Additionally, our expenses could increase if we are required by the FDA, Health Canada, the EMA or any other comparable foreign regulatory authority to perform clinical trials in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of AMX0035 in additional indications. It is possible that even if AMX0035 or any future product candidate has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of AMX0035 or any future product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials we may fail to detect toxicity of or intolerability caused by AMX0035 or any future product candidate, or mistakenly believe that AMX0035 or any future product candidates are toxic or not well-tolerated when that is not in fact the case.

AMX0035 and any of our future product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA, Health Canada, the EMA or other or comparable foreign regulatory authorities may disagree as to the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA, Health Canada, the EMA or other comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication and, if necessary, that a product candidate and any active components thereof are safe and effective for the proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA, Health Canada, the EMA or other comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from clinical trials or preclinical studies;
- the data collected from clinical trials of AMX0035 or any future product candidates may not be sufficient to support the submission of an NDA or other submission to the FDA or to obtain regulatory approval in Canada, the United States, the EU or elsewhere;
- the FDA, Health Canada, the EMA or other comparable foreign regulatory authorities may find deficiencies with clinical trial sites or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA, Health Canada, the EMA or other comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of clinical trial results may result in our failing to obtain regulatory approval to market AMX0035 or any future product candidate we develop, which would significantly harm our business, results of operations and prospects. There is no assurance that the endpoints and trial designs used for the approval of currently approved drugs for the treatment of neurodegenerative diseases will be acceptable for future approvals, including for AMX0035. The FDA, Health Canada, the EMA and other comparable foreign authorities have substantial discretion in the approval process, and determining when or whether regulatory approval will be obtained for any product candidate that we develop. Even if we believe the data collected from future clinical trials of AMX0035 or any future product candidates are promising, such data may not be sufficient to support approval by the FDA or any other regulatory authority. For example, although we

believe that the results of our CENTAUR trial demonstrate that the administration of AMX0035 resulted in a statistically significant improvement of both long-term function, as measured by the ALSFRS-R score, and survival (based on a longer-term analysis of CENTAUR patients), regulatory authorities may request additional clinical data.

The FDA reviews an NDA to determine whether the product is safe and effective for its intended use(s), with the latter determination being made on the basis of substantial evidence. The FDA has interpreted this evidentiary standard to require typically at least two adequate and well-controlled clinical investigations to establish effectiveness of a drug product, although under certain circumstances FDA has indicated that a single multi-center trial with certain characteristics, or one adequate and well-controlled trial with confirmatory evidence, may also satisfy this standard. Nonetheless, the FDA generally requires two adequate and well controlled Phase 3 clinical trials demonstrating safety and efficacy before granting marketing approval of a drug product. Accordingly, there is no guarantee that FDA will grant marketing approval to AMX0035 on the basis of the CENTAUR trial. Even if the FDA accepts our NDA submission, they may issue a Complete Response Letter if they otherwise deem our NDA submission to be deficient for approval purposes which would delay our plans for commercialization even if we are not required to conduct additional trials.

There can be no assurance that the FDA and other regulatory agencies, including Health Canada and the EMA, will not require additional clinical trials to support an application for the use of AMX0035 in the treatment of ALS or any other indication. This may be the case particularly as these regulatory authorities may consult with one another or as we may be required to apprise the respective agencies of studies we are conducting of AMX0035 for ALS in conjunction with our requests for marketing approval or in response to requests and updates from the respective agency. Thus, Health Canada and the EMA may also find that our CENTAUR trial, together with any data from our global Phase 3 PHOENIX trial that may be provided during the review period for these applications, is not sufficient to support our request for marketing authorization in those jurisdictions. It is typically the case not just in the United States, but also in Canada and Europe, that marketing approvals are based on two Phase 3 clinical studies.

In addition, disruptions caused by the COVID-19 pandemic may increase the likelihood that we encounter difficulties or delays in initiating, screening, enrolling, conducting, or completing our ongoing and planned preclinical studies and clinical trials. Clinical site initiation and patient screening and enrollment may be delayed due to prioritization of hospital resources toward the COVID-19 pandemic. Investigators and patients may not be able to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services. Similarly, our ability to recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19, could be limited, which in turn could adversely impact our clinical trial operations. Additionally, we may experience interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic. As a result of the COVID-19 pandemic, we may face delays in meeting our anticipated timelines for our ongoing and planned clinical trials.

Additionally, as of May 26, 2021, the FDA noted it is continuing to ensure timely reviews of applications for medical products during the ongoing COVID-19 pandemic in line with its user fee performance goals and conducting mission critical domestic and foreign inspections to ensure compliance of manufacturing facilities with FDA quality standards. However, the FDA may not be able to continue its current pace and approval timelines could be extended, including where a pre-approval inspection or an inspection of clinical sites is required and due to the ongoing COVID-19 pandemic and travel restrictions the FDA is unable to complete such required inspections during the review period.

Since March 2020, when foreign and domestic inspections were largely placed on hold due to the COVID-19 pandemic, the FDA has been working to resume routine surveillance, bioresearch monitoring and pre-approval inspections on a prioritized basis. The FDA has developed a rating system to assist in determining when and where it is safest to conduct prioritized domestic inspections. As of May 2021, certain inspections, such as foreign preapproval, surveillance, and for-cause inspections that are not deemed mission-critical, remain temporarily postponed. In April 2021, the FDA issued guidance for industry formally announcing plans to employ remote interactive evaluations, using risk management methods, to meet user fee commitments and goal dates and in May 2021 the FDA announced plans to continue progress toward resuming standard operational levels. Should the FDA determine that an inspection is necessary for approval and an inspection cannot be completed during the review cycle due to restrictions on travel, and the FDA does not determine a remote interactive evaluation to be adequate, the agency has stated that it generally intends to issue a complete response letter or defer action on the application until an inspection can be completed. Further, if there is inadequate information to make a determination on the acceptability of a facility, the FDA may defer action on the application until an inspection can be completed. In 2020 and 2021, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic and may experience delays in their regulatory activities. For example, with respect to new sites or facilities in the European Economic Area, or EEA, which have never had a current Good Manufacturing Practices, or cGMP, inspection or authorization, the EMA has stated that a distant assessment may be conducted in order to evaluate if the site could be authorized without an on-site pre-approval inspection. If an approval is granted, it should be indicated that the certificate has been granted on the basis of a distant assessment and an on-site inspection should be conducted when circumstances permit. If a cGMP certificate cannot be granted as a result of the distant assessment, a clock-stop in the regulatory approval process will be imposed until an on-site inspection is possible. In addition, even if we were to obtain approval, regulatory authorities may approve any of AMX0035 or any future product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for AMX0035 or any future product candidates.

In Canada, pre-approval GMP inspections are not performed in association with the NDS. Instead, Health Canada relies on a Drug Establishment License, or DEL, to determine the site's compliance with GMP. DELs can only be held by companies in Canada, and that company becomes the importer of record for the drug. To import, the sites of manufacture, testing and packaging of the Drug Substance and Drug Product are required to be listed on the DEL. Listing is dependent on having an inspection report from a recognized sister regulatory agency such as the EMA or the FDA. As a result of the COVID-19 pandemic, inspection reports can now be up to three years old. The site of manufacture of the drug product for AMX0035 is in Canada and is subject to routine inspections from Health Canada. These Canadian inspections are currently being performed remotely as a result of the COVID-19 pandemic.

We may incur unexpected costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of AMX0035 or any future product candidates.

To obtain the requisite regulatory approvals to commercialize any of AMX0035 and any future product candidates, we must demonstrate through extensive preclinical studies and clinical trials that such product candidates are safe and effective in humans. Clinical testing is expensive and can take

many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process and our future clinical trial results may not be successful.

We may experience delays in completing our clinical trials or preclinical studies and initiating or completing additional clinical trials. We may also experience numerous unforeseen events during our clinical trials that could delay or prevent our ability to receive marketing approval or commercialize AMX0035 or any future product candidates we develop, including:

- regulators, or institutional review boards, or IRBs, or other reviewing bodies may not authorize us or our investigators to commence a clinical trial, or to conduct or continue a clinical trial at a prospective or specific trial site;
- we may not reach agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the number of subjects or patients required for clinical trials of AMX0035 in an indication or any future product candidate may be larger than we anticipate, enrollment in these clinical trials may be insufficient or slower than we anticipate, and the number of clinical trials being conducted at any given time may be high and result in fewer available patients for any given clinical trial, or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors, including those manufacturing AMX0035 or any future product candidates or conducting clinical trials on our behalf, may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may have to amend clinical trial protocol submitted to regulatory authorities or conduct additional studies to reflect changes in regulatory requirements or guidance, which we may be required to resubmit to an IRB and regulatory authorities for re-examination;
- unforeseen safety events may occur during the course of a clinical trial and these events may result in the temporary suspension or termination of a clinical trial, or require urgent safety measures or restrictions to protect human subjects during the conduct of a clinical trial;
- regulators, IRBs or other reviewing bodies may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we enter into agreement for clinical and commercial supplies, or the supply or quality of AMX0035 or any future product candidate or other materials necessary to conduct clinical trials of AMX0035 or any future product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and
- the potential for approval policies or regulations of Health Canada, the FDA, the EMA or any other applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Regulators, IRBs of the institutions in which clinical trials are being conducted or data monitoring committees may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Negative or inconclusive impressions of the results from our earlier clinical trials of AMX0035 for the treatment of ALS or AD or any other clinical trial or preclinical studies in animals that we have conducted, could mandate repeated or additional preclinical studies or clinical trials and could result in

changes to or delays in preclinical studies or clinical trials of AMX0035 in other indications. We do not know whether any clinical trials that we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market AMX0035 for our initial or potential additional indications, or any future product candidate. If later stage clinical trials, including our ongoing global Phase 3 PHOENIX trial in ALS, do not produce favorable results with very strong statistical significance, our ability to obtain regulatory approval for AMX0035 for ALS or potential additional indications, or any future product candidate, may be adversely impacted.

Our failure to successfully initiate and complete clinical trials of AMX0035 for ALS, AD or potential additional indications and to demonstrate the efficacy and safety of AMX0035, including each component thereof; necessary to obtain regulatory approval to market AMX0035 would significantly harm our business. Our product candidate development costs will also increase if we experience delays in testing or regulatory approvals and we may be required to obtain additional funds to complete clinical trials. We cannot assure you that our clinical trials will begin as planned or be completed on schedule, if at all, or that we will not need to restructure our trials after they have begun. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize AMX0035 or any future product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize such product candidates, which may harm our business and results of operations. In addition, many of the factors that cause, or lead to, delays of clinical trials may ultimately lead to the denial of regulatory approval of AMX0035 or any future product candidate.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us or any future collaboration partners from obtaining approvals for the commercialization of AMX0035 or maintaining any conditional authorization for our initial or potential additional indications as well as for any future product candidate we develop.

Any product candidate we may develop and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, and distribution, are subject to comprehensive regulation by Health Canada, the FDA, the EMA and other regulatory authorities in the United States and in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction and it is possible that none of the product candidates we may seek to develop in the future will ever obtain regulatory approval. We have no experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs or regulatory consultants to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the biologic product candidate's safety, purity, efficacy and potency. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we develop may not be effective, may be only moderately effective, or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, in the United States, Canada, EU and other foreign jurisdictions, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity, and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or

regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA, Health Canada, the EMA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit, or prevent marketing approval of a product candidate. For example, based on dialogue with the FDA, we made our NDA submission to the FDA without including data from our ongoing Phase 3 PHOENIX trial; however, the FDA or other regulatory authorities may disagree with our data or rationale, or both, and thus may not approve our NDA for the treatment of ALS, or may require completion of our ongoing Phase 3 PHOENIX global clinical trial prior to issuing an approval decision for our marketing applications for AMX0035. As such, we may be unable to obtain the marketing approvals we pursue and any marketing approvals we ultimately obtain, including any conditional approvals, may be limited or subject to restrictions or post-approval commitments that could render the approved product not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates, including for AMX0035 in other indications, may be harmed, and our ability to generate revenues will be materially impaired.

The results of early-stage clinical trials and preclinical studies may not be predictive of future results. Initial data in our clinical trials may not be indicative of results obtained when these trials are completed or in later stage trials.

The results of preclinical studies may not be predictive of the results of clinical trials, and the results of any early-stage clinical trials we commence may not be predictive of the results of the later-stage clinical trials. In addition, initial data in clinical trials may not be indicative of results obtained when such trials are completed. There can be no assurance that any of our clinical trials will ultimately be successful or support further clinical development of AMX0035 or any future product candidates. There is a high failure rate for drugs and biologics proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving promising results in earlier studies, and any such setbacks in our clinical development could have a material adverse effect on our business and operating results.

Additionally, we may utilize an “open-label” clinical trial design. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with AMX0035 or any future product candidates when studied in a controlled environment with a placebo or active control.

Interim topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim topline or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our reputation and business prospects.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

Patient enrollment is a significant factor in the timing of clinical trials, and the timing of our clinical trials depends, in part, on the speed at which we can recruit patients to participate in our trials, as well as completion of required follow-up periods. We may not be able to initiate or continue clinical trials for AMX0035 or any future product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials to such trial's conclusion as required by Health Canada, the FDA, the EMA or other comparable foreign regulatory authorities. Additionally, certain clinical trials for AMX0035 and any future product candidates may be focused on indications with relatively small patient populations, which may further limit enrollment of eligible patients or may result in slower enrollment than we anticipate. The eligibility criteria of our clinical trials, once established, may further limit the pool of available trial participants. For example the number of patients suffering from ALS, is small and, in some cases, has not been established with precision. If the actual number of patients with these diseases is smaller than we anticipate, we may encounter difficulties in enrolling patients in our clinical trials, thereby delaying or preventing development and approval of AMX0035 or any future product candidates. Even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. For example, ALS patients have significant mobility issues, morbidities and other complications that have historically made retention in ALS trials, more challenging. These challenges are also present with many other neurodegenerative indications, including indications for which we may run clinical trials in the future. In the past, we have had discontinuations in our clinical trials, including in our CENTAUR trial and CENTAUR-OLE trial. Discontinuations may occur in the future and could result in delays of our clinical trials and affect our ability to enroll additional patients in our clinical trials and impact the integrity of data from our clinical trials.

Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the severity of the disease under investigation, the nature of the trial protocol, the existing body of safety and efficacy data for the product candidate, the number and nature of competing treatments and ongoing clinical trials of competing therapies for the same indication, the proximity of patients to clinical sites, the eligibility criteria for the trial, the ability to adequately monitor patients during a trial, clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied, and the risk that patients will drop out of a trial before completing all site visits. There are limited patient pools from which to draw in order to complete our clinical trials in a timely and cost-effective manner, including due to the fact that the neurological diseases we target are rare.

Furthermore, our efforts to build relationships with patient communities may not succeed, which could result in delays in patient enrollment in our clinical trials.

Any negative results we may report in clinical trials of AMX0035 or any future product candidate may also make it difficult or impossible to recruit and retain patients in other clinical trials of that same product candidate. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop AMX0035 in ALS, AD and additional indications and any future product candidates, or could render further development impossible. For example, the impact of public health epidemics, such as the ongoing COVID-19 pandemic, may delay or prevent patients from enrolling or from receiving treatment in accordance with the protocol and the required timelines, which could delay our clinical trials, or prevent us or our partners from completing our clinical trials at all, and harm our ability to obtain approval for such product candidate. Further, if patients drop out of our clinical trials, miss scheduled doses or follow-up visits, or otherwise fail to follow clinical trial protocols, whether as a result of the COVID-19 pandemic and related illness or actions taken to slow the spread of COVID-19 or otherwise, the integrity of data from our clinical trials may be compromised or not accepted by Health Canada, the FDA, the EMA or other regulatory authorities, which would represent a significant setback for the applicable program. In addition, we may rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance.

Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.

As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, it is common that various aspects of the development activities, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and results. Any of these changes could cause AMX0035 or any future product candidates to perform differently and affect the results of ongoing clinical trials or other future clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, including comparability testing to bridge earlier clinical data obtained from AMX0035 produced under earlier manufacturing methods or formulations, and regulatory authorities may disagree on the interpretation of results from this testing. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of AMX0035 or any future product candidates and jeopardize our ability to commence sales and generate revenue.

AMX0035 or any future product candidate may cause undesirable side effects or have other properties that could delay or prevent its regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following regulatory approval, if obtained.

Undesirable side effects caused by AMX0035, or any future product candidate, could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA, Health Canada, the EMA or comparable foreign regulatory authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. In clinical trials of AMX0035 to date, AMX0035 has been generally well-tolerated, with most common treatment-emergent adverse events including diarrhea, nausea, constipation, headache, fatigue, proteinuria, and decreased appetite. Health Canada has additionally identified hypersalivation as an additional treatment-emergent adverse event in need of being addressed. However, there can be no guarantee that we would observe a similar tolerability profile of AMX0035 in our ongoing global Phase 3 PHOENIX clinical trial or in other future clinical trials. Many compounds that initially showed promise in clinical or earlier stage testing are later found to cause undesirable or unexpected side effects that prevented further development of the compound.

If unacceptable side effects arise in the development of AMX0035 or any future product candidates, we, the FDA, Health Canada, the EMA or comparable foreign regulatory authorities, the IRBs, or independent ethics committees at the institutions in which our trials are conducted, or the independent safety monitoring committee could suspend or terminate our clinical trials or regulatory authorities could order us to cease clinical trials or deny approval of AMX0035 or any future product candidates for any or all targeted indications. Treatment-emergent side effects that are deemed to be drug-related could also affect subject recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. Undesirable side effects in one of our clinical trials for AMX0035 in one indication could adversely affect enrollment in clinical trials, regulatory approval and commercialization of AMX0035 in other indications. Additionally, there may be negative findings regarding components of AMX0035 or future product candidates by other parties. For example, Humanitas Mirasole SpA, or Humanitas, is conducting a Phase 3 clinical trial in the EU to assess the safety and efficacy of TURSO in patients with ALS which may lead to additional findings as to the safety profile of TURSO. Any negative findings by third parties may impact the future approvability or labeling of AMX0035 or other product candidates we may develop. In addition, side effects may not be appropriately recognized or managed by the treating medical staff. We have no relationship with Humanitas. If their Phase 3 clinical trial is successful and TURSO is approved by the FDA or any other regulatory agency, TURSO may become a commercialized product competitive with AMX0035, unless our intellectual property protections and any regulatory exclusivities we possess or may possess in the future prevent such commercialization. Inadequate training in recognizing or managing the potential side effects of AMX0035 or any future product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

Bitter taste was frequently observed in our clinical trials of AMX0035. While bitter taste, by itself, does not present a safety risk for patients, it may lead to higher levels of patient non-compliance, which could have the effect of reducing the observed efficacy of AMX0035 in our clinical trials, including our ongoing global Phase 3 PHOENIX trial, or limit its commercial adoption.

Moreover, clinical trials of AMX0035 are conducted in carefully defined sets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of any future collaborator, may indicate an apparent positive effect of AMX0035 or a future product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects.

Finally, AMX0035 is a combination of TURSO and PB. PB has been approved by the FDA and other regulatory authorities for the treatment of patients with certain urea cycle disorders. It is possible that one or more of the active moieties in AMX0035 has also been approved by FDA or other regulatory authorities. Even if AMX0035 were to receive marketing approval or be commercialized, we would continue to be subject to the risks that the FDA or similar regulatory authorities could revoke approval of PB or any active moiety in AMX0035, if applicable, or that efficacy, manufacturing or supply issues could arise with PB or any active moiety in AMX0035, if applicable. This could result in our own products being removed from the market or being less commercially successful.

Increasing demand for expanded access to AMX0035 could negatively affect our reputation and harm our business.

We are developing AMX0035 for the treatment of ALS, AD and other potential future indications for which there are currently limited or no available therapeutic options. It is possible for individuals or groups to target companies with disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide access to AMX0035 or any of our future product candidates under an expanded access policy, our reputation may be negatively affected and our business may be harmed.

Recent media attention to individual patients' expanded access requests has resulted in the introduction and enactment of legislation at the local and national level referred to as "Right to Try" laws, such as the federal Right to Try Act of 2017, which are intended to allow patients access to unapproved therapies earlier than traditional expanded access programs. A possible consequence of both activism and legislation in this area may be the need for us to initiate an unanticipated expanded access program or to make AMX0035 or any future product candidates more widely available sooner than anticipated. We are a small company with limited resources and unanticipated trials or access programs could result in diversion of resources from our primary goals.

In addition, some patients who receive access to drugs prior to their commercial approval through compassionate use, expanded access programs or right to try access have life-threatening illnesses and have exhausted all other available therapies. The risk for serious adverse events in this patient population is high, which could have a negative impact on the safety profile of AMX0035 or future product candidates if we were to provide them to these patients, which could cause significant delays or an inability to successfully commercialize AMX0035 or future product candidates, which could materially harm our business. If we were to provide patients with AMX0035 under an expanded access program, we may in the future need to restructure or pause any compassionate use and/or expanded access programs in order to perform the controlled clinical trials required for regulatory approval and successful commercialization of AMX0035 or future product candidates, which could prompt adverse publicity or other disruptions related to current or potential participants in such programs.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our clinical development activities and the diseases AMX0035 is being developed to treat, and we intend to utilize appropriate social media in connection with our commercialization efforts following approval of AMX0035, if any. Social media practices in the biotechnology and biopharmaceutical industries continue to evolve and regulations and regulatory guidance relating to such use are evolving and not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business, resulting in potential regulatory actions against us, along with the potential for litigation related to off-label marketing or other prohibited activities and heightened scrutiny by the FDA, the SEC and other regulators. For example, patients may use social media channels to comment on their experience in an ongoing blinded clinical trial or to report an alleged adverse event. If such disclosures occur, there is a risk that trial enrollment may be adversely impacted, that we may fail to monitor and comply with applicable adverse event reporting obligations or that we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product candidates. There is also a risk of inappropriate disclosure of sensitive or confidential information or negative or inaccurate posts or comments about us on any social networking website. In addition, we may encounter attacks on social media regarding our company, management, AMX0035 or future product candidates. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions or incur other harm to our business.

If we fail to develop and commercialize AMX0035 for additional indications or fail to discover, develop and commercialize other product candidates, we may be unable to grow our business and our ability to achieve our strategic objectives would be impaired.

Although the development and commercialization of AMX0035 for the treatment of ALS is our current primary focus, as part of our longer-term growth strategy, we plan to evaluate AMX0035 in other indications and develop other product candidates. We intend to evaluate internal opportunities from AMX0035 or other potential product candidates, and also may choose to in-license or acquire

other product candidates as well as commercial products to treat patients suffering from neurodegenerative diseases and CNS or other disorders with significant unmet medical needs and limited treatment options. These other potential product candidates will require additional, time-consuming development efforts prior to commercial sale, including preclinical studies, clinical trials and approval by the FDA, Health Canada, the EMA and/or other applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, we cannot assure you that any such products that are approved will be manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives.

Research activities to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research activities may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete;
- product candidates that we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

If we are unsuccessful in identifying and developing additional product candidates, our potential for growth and achieving our strategic objectives may be impaired.

We may not be successful in our efforts to expand our pipeline by identifying additional indications and modifications for which to investigate AMX0035 in the future. We may expend our limited resources to pursue a particular indication or formulation for AMX0035 and fail to capitalize on product candidates, indications or formulations that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we are focused on specific indications and modifications for AMX0035. As a result, we may fail to generate additional clinical development opportunities for AMX0035 for a number of reasons, including, that AMX0035 may in certain indications, on further study, be shown to have harmful side effects, limited to no efficacy, or other characteristics that suggest it is unlikely to receive marketing approval and achieve market acceptance in such additional indications.

We plan to conduct several clinical trials for AMX0035 in parallel over the next several years, including multiple clinical trials in patients with ALS and other indications, which may make our decision as to which indication to focus on more difficult. As a result, we may forgo or delay pursuit of

opportunities with other indications that could have had greater commercial potential or likelihood of success. In addition, we plan to explore the use of AMX0035 in patients with Wolfram syndrome and other indications. However, we may focus on or pursue one or more of our target indications over other potential indications and such development efforts may not be successful, which would cause us to delay the clinical development and approval of AMX0035. Furthermore, research activities to identify additional indications for AMX0035 require substantial technical, financial, and human resources. We may not successfully develop these additional modifications for chemistry-related, stability-related, or other reasons. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development activities for specific indications may not yield any commercially viable products.

Additionally, we may pursue in-licenses or acquisitions of development-stage assets or programs, which entails additional risk to us. Identifying, selecting and acquiring promising product candidates requires substantial technical, financial, and human resources expertise. Efforts to do so may not result in the actual acquisition or license of a particular product candidate, potentially resulting in a diversion of our management's time and the expenditure of our resources with no resulting benefit.

For example, if we are unable to identify programs that ultimately result in approved products, we may spend material amounts of our capital and other resources evaluating, acquiring and developing products that ultimately do not provide a return on our investment.

Competitive products may reduce or eliminate the commercial opportunity for AMX0035 for our current or future indications. If our competitors develop technologies or product candidates more rapidly than we do, or their technologies or product candidates are more effective or safer than ours, our ability to develop and successfully commercialize AMX0035 may be adversely affected.

The clinical and commercial landscape for the treatment of ALS and other neurodegenerative diseases, including AD is highly competitive and subject to rapid and significant technological change. We face competition with respect to our current indications for AMX0035 and will face competition with respect to any future indications of AMX0035 or other drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, Humanitas Mirasole SpA is currently conducting a Phase 3 clinical trial in the EU to assess the safety and efficacy of TURSO in patients with ALS, which, if approved, may be commercialized as a competitor to AMX0035. If this study meets its clinical endpoints, this monotherapy treatment could be approved by the FDA, the EMA and other regulatory authorities, and TURSO may become a commercialized product competitive with AMX0035, unless our intellectual property protections and any regulatory exclusivities we possess or may possess in the future prevent such commercialization. There are also a number of large pharmaceutical and biotechnology companies that currently market and sell drugs or are pursuing the development of drug candidates for the treatment of the indications that we are pursuing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Several large pharmaceutical companies market FDA-approved drugs for the treatment of ALS. These drugs include: Riluzole, marketed by Sanofi-Aventis U.S. LLC, and Radicava, marketed by Mitsubishi Tanabe Pharma America, Inc. Our potential competitors include pharmaceutical and biotechnology companies, such as Biogen, Inc., Orphazyme A/S, Biohaven Pharmaceutical Holding Co Ltd., UCB S.A., Alexion Pharmaceuticals, Inc. and Apellis Pharmaceuticals, Inc., specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions.

Many of our competitors have significantly greater financial resources, established presence in the market, expertise in research and development, manufacturing, preclinical and clinical testing, obtaining regulatory approvals and reimbursement and marketing approved products than we do. Accordingly, our competitors may be more successful than we may be in obtaining regulatory approval for therapies and achieving widespread market acceptance. Our competitors' products may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render AMX0035 or any future product candidates obsolete or non-competitive before we can recover development and commercialization expenses. If AMX0035 is approved for the indications we are currently pursuing, it could compete with a range of therapeutic treatments that are in development. In addition, our competitors may succeed in developing, acquiring or licensing technologies and drug products that are more effective or less costly than AMX0035 or any future product candidates that we may develop, which could render such product candidates obsolete and noncompetitive.

If we obtain approval for AMX0035 or any other future product candidate, we may face competition based on many different factors, including the efficacy, safety and tolerability of our products, the ease with which our products can be administered, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Existing and future competing products could present superior treatment alternatives, including being more effective, safer, less expensive or marketed and sold more effectively than any products we may develop. Competitive products may make any products we develop obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. Such competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute our business plan.

In addition, our competitors may obtain patent protection, regulatory exclusivities or regulatory approval and commercialize products more rapidly than we do, which may impact future approvals or sales of any of our product candidates that receive regulatory approval. If Health Canada, the FDA or the EMA approves the commercial sale of AMX0035 or any future product candidate, we will also be competing with respect to marketing capabilities and manufacturing efficiency. We expect competition among products will be based on product efficacy and safety, the timing and scope of regulatory approvals, availability of supply, marketing and sales capabilities, product price, reimbursement coverage by government and private third-party payors, regulatory exclusivities and patent position. Our profitability and financial position will suffer if our product candidates receive regulatory approval, but cannot compete effectively in the marketplace.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites, as well as in acquiring technologies complementary to, or necessary for, our activities.

Off-label use for the treatment of ALS of sodium phenybutyrate, or PB, which is available as a generic drug, along with the potential sale in some jurisdictions of TURSO, which preparations are of unknown identity and may not be legally sold for the treatment of ALS, expose us to additional risks that could reduce or eliminate the commercial opportunity for AMX0035.

We are developing AMX0035 as a combination of TURSO and PB. PB has been approved by the FDA and other regulatory authorities for the treatment of patients with certain urea cycle disorders.

TURSO is being marketed in preparations of unknown identity and without approval for the treatment of ALS in some jurisdictions, including the United States. We face the risk that healthcare professionals may prescribe PB for the treatment of ALS and recommend that patients obtain a commercial preparation of TURSO not labeled or marketed for the treatment of ALS on the belief that this combination could replicate the benefits of AMX0035. Patient-directed treatment with TURSO for ALS may also arise in certain jurisdictions if the Phase 3 clinical trial to assess the safety and efficacy of TURSO in patients with ALS conducted by Humanitas Mirasole SpA in the EU reports positive results. While these practices are not recommended by the medical community and have not been approved by any regulatory authority, they may nonetheless impact our sales of AMX0035, if approved, and/or public perception of AMX0035 in the United States or abroad.

If the FDA, Health Canada, the EMA or other or comparable foreign regulatory authorities approve generic versions of AMX0035 or any future product candidate of ours that receives regulatory approval, or such authorities do not grant our products appropriate periods of non-patent exclusivity before approving generic versions of such products, the sales of such products could be adversely affected.

In the United States, once an NDA is approved, the product covered thereby becomes a "reference listed drug" in the FDA's publication, "*Approved Drug Products with Therapeutic Equivalence Evaluations*," or the Orange Book. Manufacturers may seek approval of generic versions of reference listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States. In support of an ANDA, a generic manufacturer generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration, and adequate labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning, in part, that it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Moreover, many states allow or require substitution of therapeutically equivalent generic drugs at the pharmacy level even if the branded drug is prescribed. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug may be lost to the generic product.

The FDA may not finally approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity, or NCE. For the purposes of this provision, an NCE is a drug that contains no active moiety that has previously been approved by the FDA in any other NDA. An active moiety is the molecule or ion responsible for the physiological or pharmacological action of the drug substance. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the listed drug is invalid, unenforceable or will not be infringed by the generic product. In that case, the applicant may submit its application four years following approval of the listed drug and seek to launch its generic product even if we still have patent protection for our product unless an infringement suit is timely filed by the NDA or patent holder in which case the FDA cannot approve the ANDA for 30 months unless a court decision in favor of the generic manufacturer is issued earlier. For fixed dose combination products, the FDA has taken the position that a combination product will be eligible for NCE exclusivity (also known as data exclusivity) if it contains a new active moiety, even if the fixed-combination also contains a drug substance with a previously approved active moiety.

The FDA may determine, however, that AMX0035 is not eligible for NCE exclusivity, if and when FDA approves an NDA for the product. For example, even under its fixed dose combination product policies, the FDA may find that the active moieties in AMX0035 have been previously approved and,

therefore, NCE exclusivity is not available for AMX0035. The regulatory authorities in Canada and Europe may reach the same conclusions as the FDA since the determination of data exclusivity for new drug products in those jurisdictions is very similar to that of the United States.

If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval, subject to the requirement that the ANDA applicant certifies to the invalidity or non-infringement of any patents listed for our products in the Orange Book. If an infringement suit is timely filed by the NDA or patent holder, the FDA cannot finally approve the ANDA for 30 months unless a court decision in favor of the generic manufacturer is issued earlier. Three-year exclusivity is given to a drug if it contains an active moiety that has previously been approved, and the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the NDA. This form of data exclusivity is known as New Clinical Investigation, or NCI, exclusivity. If AMX0035 is approved with only NCI exclusivity, generic manufacturers may file their ANDAs anytime following approval of AMX0035 and seek to launch their generic products following the expiration of the three year market exclusivity period, even if we still have patent protection for our product.

In addition, in the United States the FDCA provides a period of seven years of orphan drug exclusivity for drugs that treat small patient populations less than 200,000 patients or for which there are more than 200,000 patients but there is no reasonable expectation that the cost of developing and making the drug for such disease or condition will be recovered from sales in the United States of such drug. If AMX0035 is granted orphan drug exclusivity, the FDA cannot finally approve a generic or a brand product that contains the same active moiety for the same orphan indication as AMX0035, for a period of seven years, subject to certain exceptions.

In Canada, we were notified that Health Canada preliminarily regards AMX0035 as a New Active Substance. However, a final determination is not made until the date of a potential Notice of Compliance (approval), or NOC. Once defined as a New Active Substance, the drug product is given eight years of data exclusivity. For the first six years, a generic product cannot be filed. After six years, a generic product can be filed, but the NOC cannot be granted for eight years. There is no regulatory provision in Canada that provides orphan drug exclusivity to approved products for rare diseases.

In the EU, innovative medicinal products (including both small molecules and biological medicinal products), sometimes referred to as new active substances, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents generic or biosimilar applicants from referencing the innovator's preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization, for a period of eight years from the date on which the reference product was first authorized in the EU. During the additional two-year period of market exclusivity, a generic or biosimilar marketing authorization can be submitted, and the innovator's data may be referenced, but no generic or biosimilar product can be marketed until the expiration of the market exclusivity period. This 10-year marketing exclusivity period may be extended to 11 years if, during the first eight of those 10 years, the marketing authorization holder obtains an approval for one or more new therapeutic indications that bring significant clinical benefits compared with existing therapies. However, even if an innovative medicinal product gains the prescribed period of data exclusivity, another company may market another version of the product if such company obtained a marketing authorization based on an application with a complete and independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Competition that AMX0035 or any future products, if approved, may face from generic versions of such products could negatively impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on our investments in those product candidates.

We currently have limited marketing, sales or distribution infrastructure. If we are unable to fully develop our sales, marketing and distribution capability on our own or through collaborations with marketing partners, we may not be successful in commercializing our product candidates.

We are currently building our marketing, sales or distribution capabilities. We have not commercialized or marketed any products to date. If AMX0035 is approved for the treatment of ALS, AD, or other future indications, we will need to expand our sales and marketing organization, either on our own or in collaboration with third parties, and add further technical expertise and supporting distribution capabilities to commercialize the approved product in key territories, which will require substantial additional resources. Some or all of these costs may be incurred in advance of any approval of AMX0035. Any failure or delay in the development of our or third parties' internal sales, marketing and distribution capabilities would adversely impact the commercialization of AMX0035 and other future product candidates.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with building out an independent sales and marketing organization.

With respect to our existing and future product candidates, we may choose to collaborate with third parties that have direct sales forces and established distribution systems to serve as an alternative to our own sales force and distribution systems. Our product revenue may be lower than if we directly marketed or sold our products, if approved. In addition, any revenue we receive will depend in whole or in part upon the efforts of these third parties, which may not be successful and are generally not within our control. If we are not successful in commercializing any approved products, our future product revenue will suffer and we may incur significant additional losses.

If we do not expand our sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing AMX0035 or any future product candidates.

Any of our current and future product candidates for which we, or any future collaborators, obtain regulatory approval in the future will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. If approved, AMX0035 and any future product candidates could be subject to post-marketing restrictions or withdrawal from the market and we, or any future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

AMX0035 or any future product candidates for which we, or any future collaborators, obtain regulatory approval, as well as the manufacturing processes, post-approval studies, labeling,

advertising and promotional activities for such product, among other things, will be subject to ongoing requirements of and review by the FDA, Health Canada, the EMA and other applicable regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. For certain commercial prescription drug products, manufacturers and other parties involved in the supply chain must also meet chain of distribution requirements and build electronic, interoperable systems for product tracking and tracing and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or other products that are otherwise unfit for distribution in the United States. We and our contract manufacturers will also be subject to user fees and periodic inspection by regulatory authorities to monitor compliance with these requirements and the terms of any product approval we may obtain. Even if regulatory approval of a product candidate is granted, the approval may be subject to limitations on the indications or uses for which the product may be marketed or to the conditions of approval, including the requirement in the United States to implement a Risk Evaluation and Mitigation Strategy, or REMS.

The FDA, Health Canada, the EMA and other regulatory authorities may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. For example, the FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. Regulatory authorities impose stringent restrictions on manufacturers' communications regarding off-label use. However, companies generally may share truthful and not misleading information that is otherwise consistent with a product's approved labeling. If we, or any future collaborators, do not market AMX0035 or any of our future product candidates for which we, or they, receive regulatory approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing if it is alleged that we are doing so. Violation of laws and regulations relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws, including the False Claims Act and any comparable foreign laws. In the EU, the direct-to-consumer advertising of prescription-only medicinal products is prohibited. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public, and may also impose limitations on our promotional activities with health care professionals.

Post-marketing requirements in Canada are similar to those in the United States. If an NDS is approved, Health Canada will require that we submit a Risk Management Plan, or RMP. Health Canada may, as part of the RMP, require that we use a registry or limited distribution channels or conduct additional clinical studies. Standard pharmacovigilance activities will also be required for any approved NDS. Any labelling changes or changes in the product supply chain would need to be submitted and approved by Health Canada. Advertising will be monitored, and will routinely be reviewed by the Pharmaceutical Advertising Advisory Board. Reimbursement in Canada is complex and will require submissions to both public and private payors to gain access to prescription drug formulary lists. In addition, if there are any patents associated with AMX0035, the product will be subject to the Patented Medicine Prices Review Board, or PMPRB.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on the manufacturing of such products;

- restrictions on the labeling or marketing of such products;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- exclusion from federal health care programs such as Medicare and Medicaid;
- suspension or withdrawal of regulatory approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Obtaining and maintaining regulatory approval of AMX0035 or any future product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of those product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of AMX0035 and any future product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if a regulatory authority, such as Health Canada, grants marketing approval of AMX0035, comparable regulatory authorities in the United States, EU and other foreign jurisdictions must also approve the manufacturing, marketing and promotion of AMX0035 in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in Canada, the United States or the EU including additional preclinical studies or clinical trials, as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States including Canada and certain jurisdictions in the EU, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

We have submitted marketing applications in the United States and Canada, and plan to submit a marketing application in the EU in the first quarter of 2022. Regulatory authorities in jurisdictions outside of the United States have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions and such regulatory requirements can vary widely from country to country. Obtaining other regulatory approvals and compliance with other regulatory requirements could result in significant delays, difficulties and costs for us and could require additional preclinical studies or clinical trials, which could be costly and time-consuming and could delay or prevent the introduction of our products in certain countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in

obtaining regulatory approval in either domestic or international markets. If we fail to comply with the regulatory requirements in international markets and/or obtain and maintain applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of AMX0035 or any future product candidates will be harmed.

Even though we have obtained orphan drug designation for AMX0035 for the treatment of ALS in the United States and EU and for the treatment of Wolfram syndrome in the United States, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.

Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the United States, or a patient population of greater than 200,000 people in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the EU, the EMA Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the EU. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the drug or biologic product. In either case, the applicant for orphan designation must also demonstrate that no satisfactory method of diagnosis, prevention, or treatment for the condition has been authorized (or, if such a method exists, the new product would be a significant benefit to those affected compared to the product available).

In September 2017, the FDA granted orphan drug status to AMX0035 for the treatment of patients with ALS in the United States, and in June 2020, the EMA granted orphan drug status to AMX0035 for the treatment of patients with ALS in the EU. We also received orphan drug status for AMX0035 for the treatment of patients with Wolfram syndrome in the United States in November 2020. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for that time period. Another drug may receive marketing approval prior to AMX0035. The applicable period is seven years in the United States and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory agency's agreed upon pediatric investigation plan. The exclusivity period in the EU can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. In the EU, during the ten-year period of orphan marketing exclusivity, neither the competent authorities of the EU Member States, the EMA, or the European Commission are permitted to accept applications or grant marketing authorization for other similar medicinal products. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA and the EMA can subsequently approve another drug for the same condition before the expiration of the seven-year (or ten-year in the EU) exclusivity period if the FDA or the EMA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In addition, if an orphan designated product receives

marketing approval for an indication broader than or different from what is designated, such product may not be entitled to orphan exclusivity. Even though the FDA has granted orphan drug designation to AMX0035 for the treatment of ALS and Wolfram syndrome, if we receive approval for AMX0035 for a modified or different indication, our current orphan designations may not provide us with exclusivity.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process. Also, regulatory approval for any product candidate may be withdrawn, and other product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

Even if we obtain orphan drug exclusivity for AMX0035, that exclusivity may not effectively protect us from competition because different drugs can be approved for the same condition before the expiration of the orphan drug exclusivity period. For example, even if orphan drug exclusivity is granted to AMX0035 if and when it is approved, that exclusivity may not prevent the approval, of TURSO by the FDA, the EMA or other regulatory authorities as a monotherapy treatment for ALS if those regulatory agencies determine that TURSO is a different drug product from AMX0035. In addition, the regulatory authorities may find that this monotherapy treatment is clinically superior to our fixed dose product and approve it even if we are granted orphan drug exclusivity. U.S. lawmakers have also recently raised the possibility that regulatory or legislative changes might need to be made to the Orphan Drug Act to foster competition. This includes the introduction of legislation that, if adopted into law, would require us to demonstrate to the FDA that AMX0035 would be economically unviable when facing competition to maintain our exclusivity.

We may pursue orphan drug designation for AMX0035 for the treatment of additional indications. The incidence and prevalence of the target patient populations for these indications will be based on our estimates and third-party data. If the market opportunity for these target populations is larger than we estimate, we may be unable to receive orphan drug designation. Additionally, if orphan drug designation is granted, we may be unable to maintain any benefits associated with orphan drug designation, including market exclusivity.

Periodically, we make estimates regarding the incidence and prevalence of target patient populations based on various third-party sources and internally generated analysis. Our estimates may be inaccurate or based on imprecise data. As described above, under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the United States, or a patient population of greater than 200,000 people in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. If our incidence or prevalence estimates for future indications for which we may seek orphan drug designation are incorrect, we may be unable to receive orphan drug designation.

Even if the FDA grants orphan drug designation for AMX0035 for other indications, exclusive marketing rights in the United States may be limited if we seek FDA marketing approval for an indication broader than the orphan designated indication. Additionally, any product candidate that initially receives orphan drug status designation, may lose such designation if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In addition, others may obtain orphan drug status for products addressing the same diseases or conditions as products we are developing, thus limiting our ability to compete in the markets addressing such diseases or conditions for a significant period of time. As a result, our business and prospects could suffer.

We may seek Fast Track Designation by the FDA for a product candidate that we develop, and we may be unsuccessful. If we are successful, the designation may not actually lead to a faster development or regulatory review or approval process.

We may seek Fast Track Designation for future product candidates we develop. If a product is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address an unmet medical need for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may rescind the Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development activities.

We may seek Breakthrough Therapy Designation by the FDA for a product candidate that we develop, and we may be unsuccessful. If we are successful, the designation may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek Breakthrough Therapy Designation for any product candidate that we develop. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for accelerated approval and priority review.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe a product candidate we develop meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if any product candidate we develop qualifies as a breakthrough therapy, the FDA may later decide that the drug no longer meets the conditions for qualification and rescind the designation.

We may seek Priority Review Designation by Health Canada for AMX0035 or future product candidates. Even if we are successful, that designation may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

As in the United States, the regulatory framework in Canada authorizes Health Canada to grant expedited review to an NDS. Priority review is meant to reduce the time period for review of an NDS from 300 days to 180 days. To qualify for priority review in Canada, the applicant must demonstrate that the candidate product is intended for the treatment, prevention or diagnosis of serious, life-threatening or severely debilitating illnesses or conditions where (a) there is no existing drug on the Canadian market with the same profile or (b) where the new product represents a significant improvement in the benefit/risk profile over existing products.

Moreover, even if AMX0035 or any future product candidate that we may develop is granted priority review by Health Canada, that designation does not guarantee that our NDS will be reviewed on an expedited basis nor does it assure or increase the likelihood that our NDS will be approved.

Product liability lawsuits against us or any of our future collaborators could divert our resources and attention, cause us to incur substantial liabilities and limit commercialization of AMX0035 or any future product candidates.

We are exposed to potential product liability and professional indemnity risks that are inherent in the research, development, manufacturing, marketing and use of pharmaceutical products. Currently, we have no products that have been approved for commercial sale; however, the use of AMX0035 by us and any collaborators in clinical trials, and the sale of AMX0035, if approved, in the future, may expose us to liability claims. Product liability claims may be brought against us or our partners by participants enrolled in our clinical trials, patients, health care providers, pharmaceutical companies, our collaborators or others using, administering or selling any of our future approved products. If we cannot successfully defend ourselves against any such claims, we may incur substantial liabilities or be required to limit commercialization of AMX0035 or any future product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for any of our future approved products;
- injury to our reputation;
- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- significant litigation costs, including with respect to potential class action lawsuits;
- substantial monetary awards to, or costly settlements with, patients or other claimants;
- product recalls or a change in the indications for which they may be used;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize AMX0035 or any future product candidates.

Although the clinical trial process is designed to identify and assess potential side effects, clinical development does not always fully characterize the safety and efficacy profile of a new drug, and it is always possible that a drug, even after regulatory approval, may exhibit unforeseen side effects. If AMX0035 was to cause adverse side effects during clinical trials or after approval, we may be exposed to substantial liabilities. Physicians and patients may not comply with any warnings that identify known potential adverse effects and patients who should not use AMX0035 or any of our future product candidates. If any of our current or future product candidates, including AMX0035, are approved for commercial sale, we will be highly dependent upon consumer perceptions of us and the safety and quality of our products. We could be adversely affected if we are subject to negative publicity associated with illness or other adverse effects resulting from patients' use or misuse of our products or any similar products distributed by other companies.

Although we maintain product liability insurance coverage in the amount of up to $5.0 million in the aggregate, including clinical trial liability, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if we commercialize AMX0035 or any future product candidate that receives regulatory approval. In addition, insurance

coverage is becoming increasingly expensive. If we are unable to maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of AMX0035 or any future product candidates, which could harm our business, financial condition, results of operations and prospects.

Even if we, or any future collaborators, obtain regulatory approvals for AMX0035 or any future product candidate, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products, which could impair our ability to generate revenue.

Once regulatory approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and any future collaborators, must therefore comply with requirements concerning advertising and promotion for AMX0035 or any future product candidate for which we or they obtain regulatory approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and any future collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA, Health Canada and EMA requirements, including ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practices, or cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, any future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA, Health Canada and the EMA to monitor and ensure compliance with cGMPs. Despite our efforts to inspect and verify regulatory compliance, one or more of our third-party manufacturing vendors may be found on regulatory inspection by the FDA, the EMA or other authorities to be not in compliance with cGMP regulations, which may result in shutdown of the third-party vendor or invalidation of drug product lots or processes. In some cases, a product recall may be warranted or required, which would materially affect our ability to supply and market our drug products.

Accordingly, assuming we, or any future collaborators, receive regulatory approval for AMX0035 or one or more future product candidates, we, and any future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and any future collaborators, are not able to comply with post-approval regulatory requirements, we, and any future collaborators, could have the regulatory approvals for AMX0035 or any future products withdrawn by regulatory authorities and our, or any future collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Laws and regulations governing any international operations we expect to have in the future may preclude us from developing, manufacturing and selling certain products outside of the United States and will require us to develop and implement costly compliance programs.

We expect to engage in operations outside of the United States, including in Canada and in the EU initially, as well as other potential jurisdictions, and we must dedicate additional resources to

comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring us to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders, including export control and trade sanctions laws, also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Risks Related to Our Dependence on Third Parties

We may seek to establish collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.

The advancement of AMX0035, and any future product candidates and development programs or activities, as well as the potential commercialization of AMX0035 and any future product candidates will require substantial additional cash to fund expenses. For some indications of AMX0035 or future product candidates, we may decide to collaborate with additional pharmaceutical and biotechnology companies with respect to development and potential commercialization. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. In addition, if we are able to obtain regulatory approval for product candidates from foreign regulatory authorities, we may enter into collaborations with international biotechnology or pharmaceutical companies for the commercialization of such product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, design or results of clinical trials, the likelihood of approval by Health Canada, the FDA, the EMA or other comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available for collaboration and whether such a collaboration could be more attractive than the one with us for our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop AMX0035 or any future product candidates or bring them to market and generate product revenue.

Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Any collaboration agreements that we enter into in the future may contain restrictions on our ability to enter into potential collaborations or to otherwise develop specified product candidates. We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs or activities, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense.

We may in the future enter into collaborations with third parties for the development and commercialization of AMX0035 or any future product candidates, and our prospects with respect to those product candidates will depend in significant part on the success of those collaborations.

We may rely on collaborations for the development and commercialization of AMX0035 and any future product candidates. For example, we may utilize a variety of distribution, collaboration and other

marketing arrangements with one or more third parties to facilitate commercialization of AMX0035. Our likely collaborators for any distribution, development, sales, marketing, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into such collaborations, we may have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of AMX0035 or any future product candidates. Our ability to generate revenues from these arrangements will depend on any future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving AMX0035 and any future product candidates pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of AMX0035 or any future product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with AMX0035 or any of our future product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, including trade secrets and intellectual property rights, contract interpretation, or the preferred course of development might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours is involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

We rely on third parties to assist in conducting our clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or commercialize AMX0035 or any future product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.

We have relied upon and plan to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials and expect to rely on these third parties to conduct clinical trials of any future product candidate that we develop. Any of these third parties may terminate their engagements with us under certain circumstances. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which could negatively impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects. Clinical trials involve multiple clinical sites, vendors and other third parties and we are dependent on these vendors to ensure appropriate study conduct, statistical analysis and randomization. Errors or deviations they make in any of these activities could impact the usefulness and interpretability of clinical trial results. Clinical trials from time to time have deviations where a protocol or standard operating procedure is not perfectly carried out and where corrective actions are taken. While we may perceive these events as low risk, our perception of risk and appropriate corrective actions may differ from that of the regulators' view. Deviations from protocols or standard operating procedures during studies could result in negative regulatory opinions and outcomes.

Further, although our reliance on these third parties for clinical development activities limits our control over these activities, we remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. Moreover, the FDA, the EMA and competent authorities of the EU Member States require us to comply with Good Clinical Practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and IRBs. If we or our third-party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or the EMA may require us to perform additional clinical trials before approving AMX0035 or any future product candidates, which would delay the regulatory approval process. We cannot be certain that, upon inspection, the FDA or the EMA will determine that any of our clinical trials comply with GCPs. For example, our clinical trial sites and investigators have in the past and may in the future engage in protocol deviations which could impact the overall interpretability of the outcomes of our clinical trials. We are also required to register certain clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, the third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development activities. These contractors may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could impede their ability to devote appropriate time to our clinical activities. If these third parties, including clinical investigators, do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, clinical data necessary for regulatory approvals for AMX0035 or any future product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize AMX0035 or any future product candidates. In such an event, our financial results and the commercial prospects for AMX0035 or any future product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed.

In addition, quarantines, shelter-in-place, and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to the COVID-19 pandemic or other infectious diseases could impact personnel at our CROs, which could disrupt our clinical timelines, which could have a material adverse impact on our business, prospects, financial condition, and results of operations.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or regulatory approval of AMX0035 or any future product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue.

Our use of third parties to manufacture AMX0035 may increase the risk that we will not have sufficient quantities of AMX0035, products, or necessary quantities of such materials on time or at an acceptable cost.

We do not own or operate manufacturing facilities for the production of clinical or commercial quantities of AMX0035, and we currently lack the resources and the capabilities to do so. As a result, we currently rely on third parties for the manufacture and supply of the active pharmaceutical ingredients, or APIs, in AMX0035, and for the blending and packaging of AMX0035. Our current strategy is to outsource all manufacturing of AMX0035 and any future product candidates to third parties.

We currently engage third-party manufacturers to provide the APIs of AMX0035 and for the final drug product formulation of AMX0035 that is being used in our clinical trials and we engage separate third-parties for the blending and packaging of finished clinical materials. We must be able to demonstrate comparability of drug substance across suppliers along with stability data across suppliers. We currently rely on a single manufacturer to supply one of our APIs and a separate manufacturer to supply the other. Although we believe that there are several potential alternative manufacturers who could manufacture each of the APIs in AMX0035, we may incur added costs and delays in identifying and qualifying any such replacement. In addition, we typically order raw materials and services on a purchase order basis and do not enter into long-term dedicated capacity or minimum supply arrangements with any commercial manufacturer. For example, the extent to which the COVID-19 pandemic impacts our ability to procure sufficient supplies for the development of AMX0035, and any future products and product candidates will depend on the severity and duration of the spread of the virus, and the actions undertaken to contain COVID-19 or treat its effects. There is no assurance that we will be able to timely secure needed supply arrangements on satisfactory terms, or at all. Our failure to secure these arrangements as needed could have a material adverse effect on our ability to complete the development of AMX0035 or any future product candidates or, to commercialize them, if approved. We may be unable to conclude agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms. There may be difficulties in scaling up to commercial quantities and formulation of AMX0035, and the costs of manufacturing could be prohibitive.

Even if we are able to establish and maintain arrangements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the failure of the third-party manufacturer to comply with applicable regulatory requirements and reliance on third-parties for manufacturing process development, regulatory compliance and quality assurance;
- manufacturing delays if our third-party manufacturers give greater priority to the supply of other products over AMX0035 or any future product candidates or otherwise do not satisfactorily perform according to the terms of the agreement between us;
- limitations on supply availability resulting from capacity and scheduling constraints of third-parties;

- the possible breach of manufacturing agreements by third-parties because of factors beyond our control;
- the possible termination or non-renewal of the manufacturing agreements by the third-party, at a time that is costly or inconvenient to us; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

If we do not maintain our key manufacturing relationships, or if any of our contract manufacturers fail to perform their obligations, we may fail to find replacement manufacturers or develop our own manufacturing capabilities, which could delay or impair our ability to obtain regulatory approval for our products. If we do find replacement manufacturers, we may not be able to enter into agreements with them on terms and conditions favorable to us and there could be a substantial delay before new facilities could be qualified and registered with the FDA, Health Canada, the EMA and other foreign regulatory authorities.

Any change in manufacturer may also involve changes in manufacturing procedures and processes, which could require that we conduct bridging studies between our prior clinical supply used in our clinical trials and that of any new manufacturer. In addition, we will need to verify that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA, Health Canada, the EMA or another regulatory authority. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials.

In some cases, the technical skills required to manufacture AMX0035, or any future products or product candidates may be unique or proprietary to the original contract manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. Furthermore, a contract manufacturer may possess or acquire technology related to the manufacture of AMX0035 or any future product candidate that such contract manufacturer owns independently. This would increase our reliance on such contract manufacturer or require us to obtain a license from such contract manufacturer in order to have another contract manufacturer manufacture AMX0035 or any future product candidates. If AMX0035 for any of our initial or potential additional indications or any future product candidate is approved by any regulatory agency, we intend to utilize arrangements with third-party contract manufacturers for the commercial production of those products. This process is difficult and time consuming and we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under cGMPs that are capable of manufacturing and AMX0035 or any future product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization.

Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, seizures or voluntary recalls of AMX0035 or any future product candidates, operating restrictions and criminal prosecutions, any of which could significantly affect supplies of AMX0035 or any future product candidates. The facilities used by our contract manufacturers to manufacture AMX0035 or any future product candidates must be evaluated by the FDA, Health Canada, the EMA and certain other foreign regulatory authorities. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with cGMPs. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA, Health Canada, the EMA or others, we may not be able to secure and/or maintain regulatory approval for our product manufactured at these facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the

FDA, Health Canada, the EMA or other comparable foreign regulatory authority finds deficiencies or does not approve these facilities for the manufacture of AMX0035 or any future product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market AMX0035 or any future product candidates, if approved. Furthermore, if we are required to change contract manufacturers, we will be required to verify that the new contract manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations, which could result in further costs and delays. Contract manufacturers may face manufacturing or quality control problems causing drug substance production and shipment delays or a situation where the contractor may not be able to maintain compliance with the applicable cGMP requirements. Any failure to comply with cGMP requirements or other FDA, Health Canada, EMA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop AMX0035 or any future product candidates and market our products, if approved.

The FDA, Health Canada, the EMA and other foreign regulatory authorities require manufacturers to register manufacturing facilities. The FDA, Health Canada, the EMA and corresponding foreign regulators also inspect these facilities to confirm compliance with cGMPs. Contract manufacturers may face manufacturing or quality control problems causing drug substance production and shipment delays or a situation where the contractor may not be able to maintain compliance with the applicable cGMP requirements. Any failure to comply with cGMP requirements or other FDA, Health Canada, EMA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop AMX0035 or any future product candidates and market our products following approval.

If any third-party manufacturer of AMX0035 or any future product candidates is unable to increase the scale of its production of such product candidates, and/or increase the product yield of its manufacturing, then our costs to manufacture the product may increase and commercialization may be delayed.

In order to produce sufficient quantities to meet the demand for clinical trials and, if approved, subsequent commercialization of AMX0035, or any future product candidates that we may develop, our third-party manufacturers will be required to increase their production and optimize their manufacturing processes while maintaining the quality of the product. The transition to larger scale production could prove difficult. In addition, if our third party manufacturers are not able to optimize their manufacturing processes to increase the product yield for AMX0035 or any future product candidates, or if they are unable to produce increased amounts of such product candidates while maintaining the quality of the product, then we may not be able to meet the demands of clinical trials or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operation.

Three vaccines for COVID-19 were granted Emergency Use Authorization by the FDA in late 2020 and early 2021, and one of those later received marketing approval from the FDA. Additional vaccines may be authorized or approved in the future. The resultant demand for vaccines and potential for manufacturing facilities and materials to be commandeered under the Defense Production Act of 1950, or equivalent foreign legislation, is having rippling effects across the contract manufacturing industry, which may make it more difficult to obtain materials or manufacturing slots for the production needed for our clinical trials and, if approved, our future commercial supply, which could lead to delays in our trials and commercial distribution.

We may need to maintain licenses for active ingredients from third parties to develop and commercialize AMX0035 or a future product candidate, which could increase our development costs and delay our ability to commercialize such product candidate.

Should we decide to use API in any of AMX0035 or any future product candidates that are proprietary to one or more third parties, we would need to maintain licenses to those active ingredients from those third parties. If we are unable to gain or continue to access rights to these active ingredients prior to conducting preclinical toxicology studies intended to support clinical trials, we may need to develop alternate product candidates from these programs by either accessing or developing alternate active ingredients, resulting in increased development costs and delays in commercialization of these product candidates. If we are unable to gain or maintain continued access rights to the desired active ingredients on commercially reasonable terms or develop suitable alternate active ingredients, we may not be able to commercialize product candidates from these programs.

Risks Related to Commercialization of AMX0035 or Future Product Candidates

We currently have limited sales and marketing capabilities. If we are unable to establish effective sales and marketing capabilities or enter into agreements with third parties to market and sell AMX0035 and any future product candidates that may be approved, we may not be successful in commercializing AMX0035 and any future product candidates if and when approved, and we may be unable to generate any product revenue.

If approved, we currently intend to seek to commercialize AMX0035 in Canada, the United States and the EU directly with specialized teams, given the relative rarity of certain of the indications we are targeting. We currently have a limited marketing and sales team for the marketing, sales and distribution of AMX0035 and any future product candidates, if approved. In order to commercialize AMX0035 for the treatment of ALS, AD and other indications, if approved, or any of our future product candidates that may be approved, we must build, on a territory-by-territory basis, marketing, sales, distribution, managerial and other capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a commercial organization is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize AMX0035 or any future product candidates on our own include:

- the inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians to prescribe AMX0035 or any future product that we may develop;
- any views or opinions expressed by ALS or AD community organizations about the efficacy of AMX0035;
- the lack of complementary treatments to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- the availability of adequate coverage by and reimbursement from third-party payors; and

- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or the profitability to us from these revenue streams is likely to be lower than if we were to market and sell any product candidates that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market AMX0035 or any of our future product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties and any of them may fail to devote the necessary resources and attention to sell and market AMX0035 or any future product candidates effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we may not be successful in commercializing AMX0035 or any future product candidates.

Our efforts to educate the ALS, AD and other neurodegenerative disease medical communities and payors on the benefits of AMX0035 or any future product candidates may require significant resources given the relative rarity of certain of the indications we are targeting, and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of AMX0035 or any future product candidates, and the indications we are targeting. Even if AMX0035 or any future product candidates are approved, if we are unable to successfully market our products, we will not be able to generate significant revenues from such products, if approved.

If we are unable to expand our marketing and distribution capabilities or enter into agreements with third parties to market and sell any of AMX0035 or future product candidates for which we obtain marketing approval, we will be unable to generate any product revenue.

To successfully commercialize any products that may result from our development activities, we need to continue to expand our marketing and distribution capabilities, either on our own or with others. The development of our own marketing and distribution effort is, and will continue to be, expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaborations regarding any approved product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize AMX0035 or any future product candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of AMX0035 and any future product candidates, if approved. Without an internal team or the support of a third-party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

The market for AMX0035 for ALS, AD and other neurodegenerative diseases and for any future product candidates we may develop may be smaller than we expect.

We focus our research and product development on treatments neurodegenerative diseases. We base our market opportunity estimates on a variety of factors, including our estimates of the number of people who have these diseases, the potential scope of our approved product labels, the subset of people with these diseases who have the potential to benefit from treatment with AMX0035 or any

future product candidates, various pricing scenarios, and our understanding of reimbursement policies for rare diseases in particular countries. These estimates are based on many assumptions and may prove to be incorrect, and new studies may reduce the estimated incidence or prevalence of these diseases. Estimating market opportunities can be particularly challenging for ultra-rare indications, such as the ones we currently address, as epidemiological data is often more limited than for more prevalent indications and can require additional assumptions to assess potential patient populations. For example, as we advance AMX0035 towards commercialization, learn more about market dynamics and engage with regulators on potential marketing approvals, our view of the initial potential market opportunity for our products will become more refined. We expect the approved label to initially be directed to a narrower patient population with the opportunity to expand the label upon submission of additional clinical data. For example, we are now initially focused primarily on annual incidence of ALS. This means the initial market opportunity for AMX0035 and any future product candidates may be smaller than the total addressable market opportunity that could be achieved over time. If we are unable to advance AMX0035 or any future product candidates with attractive market opportunities, our future product revenues may be smaller than anticipated and our business may suffer. Patient identification efforts also influence the ability to address a patient population. If efforts in patient identification are unsuccessful or less impactful than anticipated, for instance, because of a lack of diagnostic initiatives, inadequate disease awareness among healthcare professionals, or otherwise, we may not address the entirety of the opportunity we are seeking. As a result, patients may be difficult to identify and access, the addressable patient population in Canada, the United States, the EU and elsewhere may turn out to be lower than expected, or patients may not be otherwise amenable to treatment with our products, all of which would adversely affect our business, financial condition, results of operations and prospects.

Even if AMX0035 or any future product candidate of ours receives regulatory approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.

We have never commercialized a product, and even if AMX0035 for the treatment of any indication, or any future product candidate of ours, is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Physicians may be reluctant to take their patients off their current medications and switch their treatment regimen to AMX0035. Further, patients often acclimate to the treatment regime that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch due to lack of coverage and adequate reimbursement. In addition, even if we are able to demonstrate our product candidates' safety and efficacy to Health Canada, the FDA, the EMA and other regulators, safety or efficacy concerns in the medical community may hinder market acceptance.

Efforts to educate the medical community and third-party payors on the benefits of our current and any future product candidates may require significant resources, including management time and financial resources, and may not be successful. If AMX0035 or any future product candidate is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of AMX0035 and any future product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to competitive therapies and our ability to successfully publicize these advantages or highlight them in any marketing materials;

- the prevalence and severity of any side effects;
- whether the product is designated under physician treatment guidelines as a first-, second- or third-line therapy;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- limitations or warnings, including distribution or use restrictions contained in the product's approved labeling;
- the strength of sales, marketing and distribution support;
- changes in the standard of care for the targeted indications for the product; and
- availability and adequacy of coverage and reimbursement from government payors, managed care plans and other third-party payors.

Any failure by AMX0035 or any other potential product candidate of ours that obtains regulatory approval to achieve market acceptance or commercial success would adversely affect our business prospects.

Healthcare insurance coverage and reimbursement may be limited or unavailable for AMX0035 and any future product candidates, if approved, which could make it difficult for us to sell any product candidates or therapies profitably.

The success of AMX0035 and any future product candidates, if approved, depends on the availability of adequate coverage and reimbursement from third-party payors. Because AMX0035 and any future product candidates represent new approaches to the treatment of the diseases they target, we cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, AMX0035 and any future product candidates or for any product that we may develop. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any such product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to any current or future product candidates we may develop (e.g., for administration of our product candidate to patients) is also important. Inadequate reimbursement for such services may lead to physician and payor resistance and adversely affect our ability to market or sell AMX0035 or any future product candidates we may develop. In addition, we may need to develop new reimbursement models in order to realize adequate value. Payors may not be able or willing to adopt such new models and patients may be unable to afford that portion of the cost that such models may require them to bear. If we determine such new models are necessary but we are unsuccessful in developing them, or if such models are not adopted by payors, our business, financial condition, results of operations and prospects could be adversely affected.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors, such as private health insurers and health maintenance organizations, are critical to new product acceptance. Government authorities and other third-party payors decide which drugs and treatments they will cover and the amount of reimbursement. Coverage and reimbursement by a

third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement from third-party payors will be obtained. There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Future coverage and reimbursement may be subject to increased restrictions, such as prior authorization requirements, both in the United States and in international markets. Orphan drugs are typically placed on the highest cost-sharing tier and a substantial percentage are subject to prior authorization requirements. Reimbursement agencies in the EU may be more conservative than CMS.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Canada, the EU and other countries has and will continue to put pressure on the pricing and usage of drug products such as AMX0035 and any future product candidates we may develop, if approved. In many countries, particularly the countries of the EU, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. For example, in Canada, price negotiations with provincial authorities can take more than 18 months before pricing and reimbursement rates are agreed-upon. Prior to these negotiations, a review by a body known as the Canadian Agency for Drugs and Technologies in Health, or CADTH, and l'Institut national d'excellence en santé et en services sociaux, or INESS, are conducted, and for patented medicines the PMPRB also has jurisdiction. Such discussions may also result in additional studies and rationale required for combination products before reimbursement will be granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product, possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the United States, the reimbursement

for AMX0035 and any future product candidates we may develop may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of product candidates. Patients are unlikely to use AMX0035 or any future product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of AMX0035 or any future product candidates. Because AMX0035 and any future product candidates may have a higher cost of goods than conventional therapies and may require long-term follow-up evaluations, the risk that coverage and reimbursement rates may be inadequate for us to achieve profitability may be greater. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for AMX0035 and any future product candidates.

Moreover, increasing efforts by governmental and other third-party payors in Canada, the EU, the United States and other foreign jurisdictions to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for AMX0035 or any future product candidates. There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. We expect to experience pricing pressures in connection with the sale of AMX0035 or any future product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, cost containment initiatives and additional legislative changes.

Ongoing healthcare legislative and regulatory reform measures may have a material adverse effect on our business and results of operations.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, or the ACA, was passed, which substantially changes the way healthcare is financed by both governmental and private insurers and significantly impacts the U.S. pharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extended the rebate program to individuals enrolled in Medicaid managed care organizations, established annual fees and taxes on manufacturers of certain branded prescription drugs and created a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% (increased from 50%, effective January 1, 2019, pursuant to the Bipartisan Budget Act of 2018) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer’s outpatient drugs to be covered under Medicare Part D.

Since its enactment, there have been numerous judicial, administrative, executive, and legislative challenges to certain aspects of the ACA, and we expect there will be additional challenges and

amendments to the ACA in the future. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Prior to the Supreme Court's decision, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is unclear how other healthcare reform measures of the Biden administration or other efforts, if any, to challenge, repeal or replace the ACA will impact our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted.

- The Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These reductions will remain in effect through 2030 unless additional action is taken by Congress. However, pursuant to the CARES Act, and subsequent legislation, these Medicare sequester reductions will be suspended from May 1, 2020 through December 31, 2021 due to the COVID-19 pandemic. Proposed legislation, if passed, would extend this suspension until the end of the pandemic.

- On May 23, 2019, CMS issued a final rule to allow Medicare Advantage plans the option to use step therapy for Part B drugs beginning January 1, 2020. This final rule codified CMS's policy change that was effective January 1, 2019.
- On May 30, 2018, the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

These laws and future state and federal healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for AMX0035 or any future product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Further, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs. CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare and review the relationship between pricing and manufacturer patient programs. At a federal level, President Biden signed an Executive

Order on July 9, 2021 affirming the administration's policy to (i) support legislative reforms that would lower the prices of prescription drug and biologics, including by allowing Medicare to negotiate drug prices, by imposing inflation caps, and, by supporting the development and market entry of lower-cost generic drugs and biosimilars; and (ii) support the enactment of a public health insurance option. Among other things, the Executive Order also directs the Department of Health and Human Services, or HHS, to provide a report on actions to combat excessive pricing of prescription drugs, enhance the domestic drug supply chain, reduce the price that the Federal government pays for drugs, and address price gouging in the industry; and directs the FDA to work with states and Indian Tribes that propose to develop section 804 Importation Programs in accordance with the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, and the FDA's implementing regulations. FDA released such implementing regulations on September 24, 2020, which went into effect on November 30, 2020, providing guidance for states to build and submit importation plans for drugs from Canada. On September 25, 2020, CMS stated drugs imported by states under this rule will not be eligible for federal rebates under Section 1927 of the Social Security Act and manufacturers would not report these drugs for "best price" or Average Manufacturer Price purposes. Since these drugs are not considered covered outpatient drugs, CMS further stated it will not publish a National Average Drug Acquisition Cost for these drugs. If implemented, importation of drugs from Canada may materially and adversely affect the price we receive for any of our product candidates. Further, on November 20, 2020 CMS issued an Interim Final Rule implementing the Most Favored Nation, or MFN, Model under which Medicare Part B reimbursement rates would have been be calculated for certain drugs and biologicals based on the lowest price drug manufacturers receive in Organization for Economic Cooperation and Development countries with a similar gross domestic product per capita. However, on August 6, 2021 CMS announced a proposed rule to rescind the Most Favored Nations rule. Additionally, on November 30, 2020, HHS published a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. Pursuant to court order, the removal and addition of the aforementioned safe harbors have been delayed until January 1, 2023. Further, implementation of these changes and new safe harbors for point-of-sale reductions in price for prescription pharmaceutical products and pharmacy benefit manager service fees are currently under review by the Biden administration and may be amended or repealed.

Additional state and federal healthcare reform measures are expected to be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for certain pharmaceutical products or additional pricing pressures.

In addition, there have been several changes to the 340B drug pricing program, which imposes ceilings on prices that drug manufacturers can charge for medications sold to certain health care facilities. On December 27, 2018, the District Court for the District of Columbia invalidated a reimbursement formula change under the 340B drug pricing program, and CMS subsequently altered the FYs 2019 and 2018 reimbursement formula on specified covered outpatient drugs. The court ruled this change was not an "adjustment" which was within the Secretary's discretion to make but was instead a fundamental change in the reimbursement calculation. However, most recently, on July 31, 2020, the U.S. Court of Appeals for the District of Columbia Circuit overturned the district court's decision and found that the changes were within the Secretary's authority. On September 14, 2020, the plaintiffs-appellees filed a Petition for Rehearing En Banc (i.e., before the full court), but was denied on October 16, 2020. On Friday July 2, 2021, the Supreme Court granted the petition. It is unclear how these developments could affect covered hospitals who might purchase our future products and affect the rates we may charge such facilities for our approved products in the future, if any.

While some of these and other proposed measures may require additional authorization to become effective, Congress and the Biden administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Governments outside the United States may impose strict price controls, which may adversely affect our revenues, if any.

In some countries, including Canada and certain Member States of the EU, the pricing of prescription drugs is, in part, subject to governmental control. Additional countries may adopt similar approaches to the pricing of prescription drugs. In such countries, pricing negotiations with governmental authorities can take considerable time after receipt of regulatory approval for a product. The EU provides options for the EU Member States to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for drug products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after coverage and reimbursement have been obtained. Reference pricing used by various countries and parallel distribution, or arbitrage between low-priced and high-priced countries, can further reduce prices. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of AMX0035 or any future product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval, which is time-consuming and costly. We cannot be sure that such prices and reimbursement will be acceptable to us. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of AMX0035 or any future product candidates in those countries would be negatively affected.

Our relationships with healthcare providers and physicians and third-party payors may be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, or FCA, which may constrain the business or financial arrangements and relationships through which such companies conduct research, sell, market and distribute pharmaceutical products. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and

promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. The applicable federal, state and foreign healthcare laws and regulations laws that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. “Remuneration” has been interpreted broadly to include anything of value. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, a person or entity can be found guilty of violating the statute without actual knowledge of the statute or specific intent to violate it. In addition, a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other;
- federal civil and criminal false claims laws and civil monetary penalty laws, including the FCA, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, false or fraudulent claims for payment to, or approval by Medicare, Medicaid or other federal healthcare programs, knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim or an obligation to pay or transmit money to the federal government, or knowingly concealing or knowingly and improperly avoiding or decreasing or concealing an obligation to pay money to the federal government. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims. The FCA also permits a private individual acting as a “whistleblower” to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose, among other things, requirements on certain covered healthcare providers, health plans and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts

to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions;

- the federal Physician Payments Sunshine Act, created under the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, these reporting obligations will extend to include transfers of value made, as well as ownership and investment interests held, during the previous year to certain non-physician providers such as physician assistants and nurse practitioners;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by nongovernmental third-party payors, including private insurers, and may be broader in scope than their federal equivalents; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts;
- U.S. lawmakers and federal and state regulatory agencies have also focused on the relationships between pharmaceutical companies and patient advocacy groups and medical organizations, with companies developing orphan drugs receiving additional scrutiny. In light of our close relationships with patient advocacy groups and healthcare professionals treating ALS and AD, we face the risk of U.S. Congressional and federal and state inquiries and investigations related to our interactions with these groups. Addressing such investigations may require substantial resources and could potentially harm our reputation; and
- European and other foreign law equivalents of each of the laws, including reporting requirements detailing interactions with and payments to healthcare providers. Much like the Anti-Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to induce or reward improper performance generally is usually governed by the national anti-bribery laws of the EU Member States, and the Bribery Act 2010 in the UK. Infringement of these laws could result in substantial fines and imprisonment. EU Directive 2001/83/EC, which is the EU Directive governing medicinal products for human use, further provides that, where medicinal products are being promoted to persons qualified to prescribe or supply them, no gifts, pecuniary advantages or benefits in kind may be supplied, offered or promised to such persons unless they are inexpensive and relevant to the practice of medicine or pharmacy. Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual EU Member States. These

requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive recordkeeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products. Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from U.S. government funded healthcare programs, such as Medicare and Medicaid, or similar programs in other countries or jurisdictions, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the delay, reduction, termination or restructuring of our operations. Further, defending against any such actions can be costly and time-consuming, and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other providers or entities with whom we expect to do business is found to not be in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment. If any of the above occur, it could adversely affect our ability to operate our business and our results of operations.

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EU, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the United States, and, as a result, increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the United States. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and or

impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater and it also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR provides that EU member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Similar actions are either in place or under way in the United States. There are a broad variety of data protection laws that are applicable to our activities, and a wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act—which went into effect on January 1, 2020—is creating similar risks and obligations as those created by GDPR, though the Act does exempt certain information collected as part of a clinical trial subject to the Federal Policy for the Protection of Human Subjects (the Common Rule). Many other states are considering similar legislation. A broad range of legislative measures also have been introduced at the federal level. Accordingly, failure to comply with federal and state laws (both those currently in effect and future legislation) regarding privacy and security of personal information could expose us to fines and penalties under such laws. There also is the threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with these requirements is rigorous and time intensive and requires significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the EU. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

Risks Related to Our Intellectual Property

Our commercial success depends on our ability to protect our intellectual property and proprietary technology.

Our commercial success depends in large part on our ability to obtain and maintain intellectual property rights protection through patents, trademarks and trade secrets in the United States and other countries with respect to our proprietary product candidate, AMX0035, and any future proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode, negate or preempt any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we have filed patent applications and may file other patent applications in the United States or abroad related to AMX0035 or any future product candidates that are important to our business; we may also license or purchase patents or patent applications filed by others. The patent application process is expensive and time-

consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

If the scope of the patent protection we obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our patents have, or that any of our pending owned patent applications that mature into issued patents will include claims with a scope sufficient to protect our proprietary therapeutics or otherwise provide any competitive advantage. Other parties have developed or may develop technologies that may be related or competitive with our approach, and may have filed or may file patent applications and may have been issued or may be issued patents with claims that overlap or conflict with our patent applications, either by claiming the same compounds, formulations or methods or by claiming subject matter that could dominate our patent position. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to AMX0035 or any future product candidates. In the event that an alternative combination, or TURSO as a single drug product, is developed and approved for use in indications for which we may seek approval and falls outside the scope of our patent claims, the marketability and commercial success of AMX0035, if approved, could be materially harmed.

Even if they are unchallenged, our owned patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapy that provides benefits similar to our product candidate but falls outside the scope of our patent protection or license rights. If the patent protection provided by the patent and patent applications we hold or pursue with respect to AMX0035 or any future product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidate could be negatively affected, which would harm our business.

We, or any future partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position.

It is possible that defects of form in the preparation or filing of our patent or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we or our partners, collaborators, or licensees whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, or licensees are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any

of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

The patent position of biotechnology and pharmaceutical companies carries uncertainty. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which are dependent upon the current legal and intellectual property context, extant legal precedent and interpretations of the law by individuals. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are characterized by uncertainty.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patent or pending patent applications, or that we were the first to file for patent protection of such inventions. If third parties have filed prior patent applications on inventions claimed in our patents or applications that were filed on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such prior applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents may be challenged in the courts or patent offices in the United States and abroad. Also, while we believe that we have disclosed all potentially relevant prior art relating to our patents and patent applications, there is no assurance that we have found all such prior art or disclosed it in every relevant jurisdiction. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivation proceedings, *ex parte* reexaminations, *inter partes* review, supplemental examinations, or interference proceedings or challenges in district court, in the United States or in various foreign patent offices, including both national and regional, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of the patent or in patent or patent application claims being narrowed, invalidated or held unenforceable, in whole or in part, or in denial of the patent application or loss or reduction in the scope of one or more claims of the patent or patent application, any of which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products.

Pending and future patent applications may not result in patents being issued that protect our business, in whole or in part, or which effectively prevent others from commercializing competitive

products. Competitors may also be able to design around our patents. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, patent laws in various jurisdictions, including jurisdiction covering significant commercial markets, such as the European Patent Office, or EPO, China and Japan, restrict the patentability of methods of treatment of the human body more than United States law does. If these developments were to occur, they could have a material adverse effect on our ability to generate revenue.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our future development partners will be successful in protecting AMX0035 or any future product candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance, whether intentional or not, can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, and sell AMX0035;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates; and
- countries other than the United States may, under certain circumstances, force us to grant a license under our patents to a competitor, thus allowing the competitor to compete with us in that jurisdiction or forcing us to lower the price of our drug in that jurisdiction.

Issued patents that we have or may obtain or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other

agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors do not infringe our patents. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

In addition, we rely on the protection of our trade secrets and proprietary, unpatented know-how. Although we have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and invention assignment agreements with employees, consultants, collaborators, vendors, and advisors, we cannot provide any assurances that all such agreements have been duly executed, and third parties may still obtain this information or may come upon this or similar information independently. It is possible that technology relevant to our business will be independently developed by a person who is not a party to such a confidentiality or invention assignment agreement. We may not be able to prevent the unauthorized disclosure or use of our technical knowledge or trade secrets by consultants, collaborators, vendors, advisors, former employees and current employees. Furthermore, if the parties to our confidentiality agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a consequence of such breaches or violations. Our trade secrets could otherwise become known or be independently discovered by our competitors. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets. If any of these events occurs or if we otherwise lose protection for our trade secrets or proprietary know-how, our business may be harmed.

It is difficult and costly to protect our intellectual property and our proprietary technologies, and we may not be able to ensure their protection.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection for our proprietary product candidate, AMX0035, as well as on successfully defending these patents against potential third-party challenges. Our ability to protect our product candidate from unauthorized making, using, selling, offering to sell or importing by third parties is dependent on the extent to which we have rights under valid and enforceable patents that cover these activities.

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved and have in recent years been the subject of much litigation. Changes in either the patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Over the past decade, U.S. federal courts have increasingly invalidated pharmaceutical and biotechnology patents during litigation often based on changing interpretations of patent law. Further, the determination that a patent application or patent claim meets all of the requirements for patentability is a subjective determination based on the application of law and jurisprudence. The ultimate determination by the U.S. Patent and Trademark Office, or USPTO, or by a court or other trier of fact in the United States, or corresponding foreign national patent offices or courts, on whether a claim meets all requirements of patentability cannot be assured. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in our owned patents or patent applications.

We cannot provide assurances that any of our patent applications will be found to be patentable, including over our own prior art publications or patent literature, or will issue as patents. Neither can we make assurances as to the scope of any claims that may issue from our pending and future patent applications nor to the outcome of any proceedings by any potential third parties that could challenge

the patentability, validity or enforceability of our patents and patent applications in the United States or foreign jurisdictions. Any such challenge, if successful, could limit patent protection for our products and current or future product candidates and/or materially harm our business.

In addition to challenges during litigation, third parties can challenge the validity of our patents in the United States using post-grant review and *inter partes* review proceedings, which some third parties have been using to cause the cancellation of selected or all claims of issued patents of competitors. For a patent filed March 16, 2013 or later, a petition for post-grant review can be filed by a third party in a nine-month window from issuance of the patent. A petition for *inter partes* review can be filed immediately following the issuance of a patent if the patent has an effective filing date prior to March 16, 2013. A petition for *inter partes* review can be filed after the nine-month period for filing a post-grant review petition has expired for a patent with an effective filing date of March 16, 2013 or later. Post-grant review proceedings can be brought on any ground of invalidity, whereas *inter partes* review proceedings can only raise an invalidity challenge based on published prior art and patents. These adversarial actions at the USPTO review patent claims without the presumption of validity afforded to U.S. patents in lawsuits in U.S. federal courts, and use a lower burden of proof than used in litigation in U.S. federal courts. Therefore, it is generally considered easier for a competitor or third party to have a U.S. patent invalidated in a USPTO post-grant review or *inter partes* review proceeding than invalidated in a litigation in a U.S. federal court. If any of our patents are challenged by a third party in such a USPTO proceeding, there is no guarantee that we will be successful in defending the patent, which may result in a loss of the challenged patent right to us.

In the EU, third parties can challenge the validity of our patents by filing an Opposition before the EPO. An adverse determination by the Opposition Board can result in the narrowing or invalidation of a European patent. If any of our European patents are challenged by a third party in such an opposition proceeding, there is no guarantee that we will be successful in defending the patent, which may result in a loss of the challenged patent right to us.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs;
- it is possible that one or more of our pending patent applications will not become an issued patent or, if issued, that the patent(s) claims will have sufficient scope to protect our technology, provide us with commercially viable patent protection or provide us with any competitive advantages;
- if our pending applications issue as patents, they may be challenged by third parties as invalid or unenforceable under United States or foreign laws;
- we may not successfully commercialize AMX0035, if approved, before our relevant patents expire;
- we may not be the first to make the inventions covered by each of our patents and pending patent applications; or
- we may not develop additional proprietary technologies or product candidates that are separately patentable.

In addition, to the extent that we are unable to obtain and maintain patent protection for AMX0035 or any future product candidates or in the event that such patent protection expires, it may no longer be cost-effective to extend our portfolio by pursuing additional development of a product or product candidate for follow-on indications.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

In addition to patents, we also may rely on trade secrets to protect our proprietary product candidate, especially where we do not believe patent protection is appropriate or obtainable. Trade secrets are difficult to protect. We seek to protect our confidential proprietary information, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators. These agreements are designed to protect our proprietary information. However, we cannot be certain that such agreements have been entered into with all relevant parties, and we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. For example, our employees, consultants, contractors, outside scientific collaborators and other advisers may unintentionally or willfully disclose our information to competitors. Enforcing a claim that a third-party entity illegally obtained and is using any of our trade secrets is expensive and time-consuming, and the outcome is unpredictable, and we may not be able to obtain adequate remedies for such breaches. We also seek to preserve the integrity and confidentiality of our confidential proprietary information by maintaining physical security of our premises and physical and electronic security of our information technology systems, but it is possible that these security measures could be breached. In addition, courts outside the United States are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. Notably, proprietary technology protected by a trade secret does not preempt the patenting of independently developed equivalent technology, even if such equivalent technology is invented subsequent to the technology protected by a trade secret. If any of our confidential proprietary information were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. The patent term of a U.S. patent may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the United States Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent.

Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a Patent Term Extension, or PTE, of up to five years beyond the normal expiration of the patent to compensate patent owners for loss of enforceable patent term due to the lengthy regulatory approval process. PTE is limited to the approved indication (or any additional indications approved during the period of extension). We anticipate applying for PTE in the United States. Similar extensions may be available in other countries where we are prosecuting patents and we likewise anticipate applying for such extensions.

The granting of such patent term extensions is not guaranteed and is subject to numerous requirement. We might not be granted an extension because of, for example, failure to apply within

applicable periods, failure to apply prior to the expiration of relevant patents or otherwise failure to satisfy any of the numerous applicable requirements. Moreover, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to obtain approval of competing products following our patent expiration by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. If this were to occur, it could have a material adverse effect on our ability to generate revenue.

Changes in the interpretation of patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

The United States Congress is responsible for passing laws establishing patentability standards. As with any laws, implementation is left to federal agencies and the federal courts based on their interpretations of the laws. Interpretation of patent standards can vary significantly within the U.S. Patent and Trademark Office, and across the various federal courts, including the Supreme Court. Recently, the Supreme Court has ruled on several patent cases, generally limiting the types of inventions that can be patented. Further, there are open questions regarding interpretation of patentability standards that the Supreme Court has yet to decisively address. Absent clear guidance from the Supreme Court, the USPTO has become increasingly conservative in its interpretation of patent laws and standards.

In addition to increasing uncertainty with regard to our ability to obtain patents in the future, the legal landscape in the United States has created uncertainty with respect to the value of patents. Depending on any actions by Congress, and future decisions by the lower federal courts and the Supreme Court, along with interpretations by the USPTO, the laws and regulations governing patents could change in unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not be able to enforce our intellectual property rights throughout the world.

Filing, prosecuting, enforcing and defending patents on our product candidate in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly in developing countries; thus, even in countries where we do pursue patent protection, there can be no assurance that any patents will issue with claims that cover our products.

Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the United States and the EU do not afford intellectual property protection to the same extent as the laws of the United States and the EU. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the United States and the EU or from selling or importing products made from our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop and market their own products

and, further, may export otherwise infringing products to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Proceedings to enforce our patent rights, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Moreover, such proceedings could put our patents at risk of being invalidated or held unenforceable, or interpreted narrowly and our pending patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products, if approved. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

Others may challenge inventorship or claim an ownership interest in our intellectual property which could expose it to litigation and have a significant adverse effect on its prospects.

A third party or former employee or collaborator may claim an inventorship or ownership interest in one or more of our or our licensors' patents or other proprietary or intellectual property rights. A third party could bring legal actions against us and seek monetary damages and/or enjoin clinical testing, manufacturing and marketing of the affected product or products. While we are presently unaware of any claims or assertions by third-parties with respect to inventorship or ownership of our patents or other intellectual property, we cannot guarantee that a third party will not assert a claim or an interest in any of such patents or intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to AMX0035 or any future product candidates. Further, regardless of the outcome, if we become involved in any litigation, it could consume a substantial portion of our resources, and cause a significant diversion of effort by our technical and management personnel.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing AMX0035 or any future product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidate without infringing the intellectual property and other proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Third parties may have U.S. and non-U.S. issued patents and pending patent applications relating to compounds, methods of manufacturing compounds and/or methods of use for the treatment of the disease indications for which we are developing AMX0035 or any future product candidates. If any third-party patents or patent applications are found to cover AMX0035 or any future product candidates or their methods of use or manufacture, we may not be free to manufacture or market such product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our products candidates,

including patent infringement lawsuits in the United States or abroad. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the composition, use or manufacture of AMX0035 or any future product candidates. While we perform periodic searches for relevant patents and patent applications with respect to our proprietary drug candidate, AMX0035, we cannot guarantee that any of our patent searches or analyses including, but not limited to, the identification of relevant patents, the scope of patent claims or the expiration of relevant patents are complete or thorough, nor can we be certain that we have identified each and every patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of AMX0035 or any future product candidates in any jurisdiction. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that AMX0035 or any future product candidates may be accused of infringing. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Accordingly, third parties may assert infringement claims against us based on intellectual property rights that exist now or arise in the future. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use or manufacture. The scope of protection afforded by a patent is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidate, product or method either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources, and we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate or product. If we were required to obtain a license to continue to manufacture or market the affected product, we may be required to pay substantial royalties or grant cross-licenses to our patents. We cannot, however, assure you that any such license will be available on acceptable terms, if at all. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations as a result of claims of patent infringement or violation of other intellectual property rights, Further, the outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance, including the demeanor and credibility of witnesses and the identity of any adverse party. This is especially true in intellectual property cases that may turn on the testimony of experts as to technical facts upon which experts may reasonably disagree. Furthermore, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively or additionally it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing AMX0035 or any future product candidates or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the

confidential information or trade secrets of third parties could have a similar negative impact on our business. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our current and former employees and our licensors' current and former employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including some which may be competitors or potential competitors. Some of these employees, including members of our senior management, may have executed proprietary rights, non-disclosure and non-competition agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may sustain damages or lose key personnel, valuable intellectual property rights or the personnel's work product, which could hamper or prevent commercialization of our technology, which could materially affect our commercial development efforts. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while we typically require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our trademarks of interest and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We rely on both registration and common law protection for our trademarks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During trademark registration proceedings, we may receive rejections. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications

and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we propose to use for our products in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed product names, we may be required to expend significant additional resources in an effort to identify a usable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Intellectual property rights do not necessarily address all potential threats to our business.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are competitive to AMX0035, for example a TURSO monotherapy, or any of our future product candidates but that are not covered by the claims of the patents that we own;
- others may independently develop similar or alternative technologies or otherwise circumvent any of our technologies without infringing our intellectual property rights;
- we or any of our collaborators might not have been the first to invent the inventions covered by the patents or patent applications that we own;
- we or any of our collaborators might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license, or will own or will have obtained a license;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- ownership of our patents or patent applications may be challenged by third parties;
- the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business; and
- patent enforcement is expensive and time-consuming and difficult to predict; thus we may not be able to enforce any of our patents against a competitor.

Our reliance on third parties for research and development and manufacturing requires us to share our trade secrets, which increases the possibility that our trade secrets will be misappropriated or disclosed, and confidentiality agreements with employees and third parties may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We consider proprietary trade secrets or confidential know-how and unpatented know-how to be important to our business. We may rely on trade secrets or confidential know-how to protect our

technology, especially where patent protection is believed by us to be of limited value. We rely on third parties for research and development work, and expect to rely on third parties for future manufacturing of our proprietary product candidate, AMX0035 and any future product candidates. We also expect to collaborate with third parties on the development of AMX0035 and any future product candidates. As a result of the aforementioned collaborations, we must, at times, share trade secrets with our collaborators.

Trade secrets or confidential know-how can be difficult to maintain as confidential. To protect this type of information against disclosure or appropriation by competitors, our policy is to require our employees, consultants, contractors and advisors to enter into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with us prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. However, current or former employees, consultants, contractors and advisers may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. The need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have an adverse effect on our business and results of operations. Enforcing a claim that a third party obtained illegally and is using trade secrets or confidential know-how is expensive, time consuming and unpredictable. The enforceability of confidentiality agreements may vary from jurisdiction to jurisdiction.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

We may need to acquire or license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development of additional future product candidates. It may be necessary for us to use the patented or proprietary technology of one or more third parties to commercialize our current and future product candidates. If we are unable to acquire such intellectual property outright, or obtain licenses to such intellectual property from such third parties when needed or on commercially reasonable terms, our ability to commercialize additional future product candidates, if approved, would likely be delayed.

The risks described elsewhere pertaining to our intellectual property rights also apply to the intellectual property rights that we may in-license, and any failure by us or our licensors to obtain, maintain, defend and enforce these rights could have an adverse effect on our business. In some cases we may not have control over the prosecution, maintenance or enforcement of the patents that we license, and may not have sufficient ability to provide input into the patent prosecution, maintenance and defense process with respect to such patents, and potential future licensors may fail to take the steps that we believe are necessary or desirable in order to obtain, maintain, defend and enforce the licensed patents.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

A pandemic, epidemic, or outbreak of an infectious disease, such as the COVID-19 pandemic, may materially and adversely affect our business, including our preclinical studies, clinical trials, third parties on whom we rely, our supply chain, our ability to raise capital, our ability to conduct regular business and our financial results.

We are subject to risks related to public health crises such as the COVID-19 pandemic and the ongoing efforts to halt its outbreak. The pandemic and policies and regulations implemented by governments in response to the pandemic, often directing businesses and governmental agencies to cease non-essential operations at physical locations, prohibiting certain nonessential gatherings and ceasing non-essential travel have also had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred, supply chains have been disrupted, facilities and production have been suspended, and demand for certain goods and services, such as medical service and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. We have experienced certain impacts of the COVID-19 pandemic to date, including having to make certain alterations to our preclinical and clinical trial activities, such as scheduling certain work off-site and performing off-site assessments. For example, we had to amend our CENTAUR trial protocol to allow for remote visits by patients, instead of patients making site visits. In addition, in some cases we were forced to delay enrollment at certain sites in our recently completed Phase 2 clinical trial for AMX0035 in AD. There can be no guarantee we will not experience other impacts, such as being forced to further delay or pause enrollment, experiencing potential interruptions to our supply chain, facing difficulties or additional costs in enrolling patients in future clinical trials or being able to achieve full enrollment of our studies within the timeframes we anticipate, or at all.

The impact of the COVID-19 pandemic has been and may continue to be extensive in many aspects of society and could continue to result in significant disruptions to the global economy, as well as businesses and capital markets around the world. The full extent to which the COVID-19 pandemic, including efforts to halt the pandemic, could ultimately impact our business, preclinical studies, clinical trials and financial results will depend on future developments, which are highly uncertain and cannot be accurately predicted, including the rate and success of vaccination efforts, new strains of the virus for which current vaccinations may not be effective, new information which may emerge, among others. Other global health concerns could also result in social, economic, and labor instability in the countries in which we or the third parties with whom we engage operate.

In response to the COVID-19 pandemic, we have continued to take precautionary measures intended to help minimize the risk of the virus to our employees, including closing or reducing access to our executive offices and temporarily requiring employees to work remotely, suspending all non-essential travel for our employees and discouraging employee attendance at industry events and in-person work-related meetings, all of which could negatively affect our business.

While we have been working closely with our third-party manufacturers, distributors and other partners to manage our supply chain activities and mitigate potential disruptions to the production of AMX0035 as a result of the COVID-19 pandemic, if, despite vaccination efforts, the COVID-19 pandemic persists for an extended period of time, there could be significant and material disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of AMX0035 and any future product candidates. Any such supply disruptions would adversely impact our ability to initiate and complete preclinical studies or clinical trials, including disruptions in procuring items that are essential for our research and development activities and securing manufacturing slots for the products needed for such activities, our ability to generate sales of and revenue from our product candidates, if approved, and our business, financial condition, results of operations and growth prospects could be materially adversely affected.

The COVID-19 pandemic has affected and may in the future affect employees of third-party CROs located in affected geographies that we rely upon to carry out our clinical trials. If current efforts to control the COVID-19 pandemic are not successful, if the spread of the virus or any variant of the virus is not contained or increases, or if a new virus or pandemic emerges, may experience additional disruptions that could severely impact our business and clinical trials, including:

- delays or difficulties in our commercialization efforts;
- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of sites or facilities serving as our clinical trial sites and staff supporting the conduct of our clinical trials, including our trained therapists, or absenteeism that reduces site resources;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal, state or national governments, employers and others or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;
- risk that participants enrolled in our clinical trials will acquire COVID-19 or another virus or illness while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events or patient withdrawals from our trials;
- limitations in employee resources that would otherwise be focused on conducting our clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;
- delays in receiving authorizations from regulatory authorities to initiate our future clinical trials;
- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials;
- interruption in global shipping that may affect the transport of clinical trial materials, such as the AMX0035 used in our clinical trials;
- changes in local regulations as part of a response to the COVID-19 pandemic or other pandemic which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or the discontinuation of the clinical trials altogether;
- interruptions or delays in preclinical studies due to restricted or limited operations at research and development laboratory facilities;
- delays in necessary interactions with local regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government employees; and
- refusal of the FDA, Health Canada, the EMA or the other regulatory bodies to accept data from clinical trials in affected geographies outside the United States, Canada or the EU or other relevant local geography.

Any negative impact the COVID-19 pandemic or any future pandemic or similar disruption has on patient enrollment or treatment or the development of AMX0035 and any future product candidates could cause costly delays to clinical trial activities, which could adversely affect our ability to obtain regulatory approval for and to commercialize AMX0035 and any future product candidates, if approved, increase our operating expenses, and have a material adverse effect on our financial results. The

COVID-19 pandemic has also in the past caused significant volatility in public equity markets and disruptions to the United States and global economies and any future pandemic or similar disruption could lead to further market dislocation. Any such increased volatility and economic dislocation may make it more difficult for us to raise capital on favorable terms, or at all. If we or any of the third parties with whom we engage were to experience renewed shutdowns or other business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively affected, which could have a material adverse impact on our business and our results of operations and financial conditions. To the extent the COVID-19 pandemic or any future pandemic or similar disruption adversely affects our business and financial results, it may also heighten many of the other risks described in this "Risk Factors" section, such as those relating to the timing and completion of our clinical trials and our ability to obtain future financing.

We depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.

Our success depends, and will likely continue to depend, upon our ability to hire, retain the services of our current executive officers, principal consultants and others, including Josh Cohen and Justin Klee, our Co-Chief Executive Officers, James Frates, our Chief Financial Officer, and Margaret Olinger, our Global Head of Commercial and Chief Commercial Officer, and Patrick Yeramian, our Global Head of Clinical Research & Development and Chief Medical Officer. We have entered into employment agreements with Mr. Cohen, Mr. Klee, Mr. Frates and Ms. Olinger and Dr. Yeramian, but they may terminate their employment with us at any time. The loss of their services might impede the achievement of our research, development and commercialization objectives.

Our ability to compete in the biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. Our industry has experienced a high rate of turnover of management personnel in recent years. Replacing executive officers or other key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize products successfully.

Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop and commercialize AMX0035 or any future product candidates will be limited.

We only have a limited number of employees to manage and operate our business.

As of October 31, 2021, we had 76 full-time employees. Our focus on the development of AMX0035 requires us to optimize cash utilization and to manage and operate our business in a highly efficient manner. We cannot assure you that we will be able to hire and/or retain adequate staffing levels to develop AMX0035 or run our operations and/or to accomplish all of the objectives that we otherwise would seek to accomplish.

Our employees, independent contractors, consultants, collaborators and CROs may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators and CROs may engage in fraudulent conduct or other illegal activity. Misconduct by those parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates:

- FDA regulations or similar regulations of comparable non-U.S. regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities;
- manufacturing standards;
- federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable non-U.S. regulatory authorities; and
- laws that require the reporting of financial information or data accurately.

Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creating fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of product materials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, disgorgement, integrity oversight and reporting obligations, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could have a material adverse effect on our ability to operate our business and our results of operations.

We expect to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of regulatory affairs and sales, marketing and distribution, as well as to support our public company operations. To manage these growth activities, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Our management may need to devote a significant amount of its attention to managing these growth activities. Moreover, our expected growth could require us to relocate to a different geographic area of the country. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion or relocation of our operations, retain key employees, or identify, recruit and train additional qualified personnel. Our inability to manage the expansion or relocation of our operations effectively may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. See "—If we fail to remediate our material weaknesses over financial reporting controls and to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations, meet our

reporting obligations or prevent fraud, and investor confidence in our company and the market price of our common stock may be materially and adversely affected." Our expected growth could also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy, including the successful commercialization of AMX0035 or any future product candidates.

Risks Related to Our Common Stock and this Offering

If you purchase shares of our common stock in this offering, you will incur immediate and substantial dilution.

The offering price of our common stock is substantially higher than the net tangible book value per share of our common stock, which on a pro forma basis was $ per share as of September 30, 2021. Based on the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, you will experience immediate dilution of $ per share, representing the difference between our pro forma net tangible book value per share after giving effect to this offering and the assumed initial public offering price. This means that you will pay a higher price per share than the amount of our total tangible assets, less our total liabilities, divided by the number of shares of common stock outstanding. Furthermore, if the underwriters exercise their over-allotment option or our previously issued options, warrant and other rights to acquire common stock at prices below the assumed initial public offering price are exercised, you will experience further dilution. In addition, you may also experience additional dilution if options or other rights to purchase our common stock that are outstanding or that we may issue in the future are exercised or converted or we issue additional shares of our common stock at prices lower than our net tangible book value at such time. For more information, see "Dilution."

We do not know whether an active, liquid and orderly trading market will develop for our common stock or what the market price of our common stock will be and as a result it may be difficult for you to sell your shares of our common stock.

Prior to this offering there has been no public market for shares of our common stock. Although we have applied to list our common stock on the Nasdaq Global Market, an active trading market for our shares may never develop or be sustained following this offering. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. The initial public offering price for our common stock will be determined through negotiations with the underwriters, and the negotiated price may not be indicative of the market price of our common stock after the offering. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price.

Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

The price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock following this offering is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our

control, including limited trading volume. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this prospectus, these factors include:

- the commencement, enrollment or results of our ongoing and future preclinical studies and clinical trials, or any future preclinical studies or clinical trials, we may conduct of AMX0035 and any future product candidates, or changes in the development status of our current and any future product candidates;
- any delay in our regulatory submissions for AMX0035 and any future product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such submissions, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results or delays in our preclinical studies and clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial, or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval for AMX0035 and any future product candidates;
- changes in laws or regulations applicable to AMX0035 and any future product candidates, including but not limited to clinical trial requirements for approvals;
- the failure to obtain coverage and adequate reimbursement of AMX0035 and any future product candidates, if approved;
- changes on the structure of healthcare payment systems;
- any changes to our relationship with any manufacturers, suppliers, licensors, future collaborators or other strategic partners;
- our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;
- our inability to establish collaborations if needed;
- our failure to commercialize AMX0035 and any future product candidates;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of AMX0035 and any future product candidates;
- introduction of new products or services offered by us or our competitors, or the release or publication of clinical trial results from competing product candidates;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- actual or anticipated variations in quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;

- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- issuances of debt or equity securities;
- sales of our common stock by us or our stockholders in the future or the perception that such sales may occur;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political and economic conditions, including the COVID-19 pandemic; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment.

We have broad discretion in the use of the net proceeds from this offering and may invest or spend the proceeds in ways with which you do not agree and in ways that may not yield a return on your investment.

Although we currently intend to use the net proceeds from this offering in the manner described in the section titled “Use of Proceeds” in this prospectus, our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. You will not have the opportunity to influence our decisions on how to use the net proceeds from this offering. The failure by our management to apply these funds effectively could result in financial losses that could harm our business, cause the price of our common stock to decline and delay the development of AMX0035 or any future product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

We are an emerging growth company and a smaller reporting company, and the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not

previously approved. We could be an emerging growth company until December 31, 2027, although circumstances could cause us to lose that status earlier, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act or if we have total annual gross revenue of $1.07 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than $1.0 billion in non-convertible debt during any three year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Investors may find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This provision allows an emerging growth company to delay the adoption of accounting standards that have different effective dates for public and private companies until those standards would otherwise apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards, and therefore we will not be subject to the same requirements to adopt new or revised accounting standards as other public companies that are not emerging growth companies.

We are also a "smaller reporting company" as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have outstanding shares of common stock based on the number of shares outstanding as of September 30, 2021. This includes the shares that we are selling in this offering, which may be resold in the public market immediately without restriction, unless purchased by our affiliates or existing stockholders. Of the remaining shares, shares are currently restricted as a result of securities laws or lock-up agreements, but will become eligible to be sold after the offering as described in the "Shares Eligible for Future Sale" section of this prospectus. Moreover, holders of an aggregate of shares of our common stock will have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. In addition, the terms of any future debt or credit agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Based upon shares outstanding as of , upon the closing of this offering, our executive officers and directors, combined with our stockholders who owned more than 5% of our outstanding common stock before this offering and their affiliates, will, in the aggregate, beneficially own shares representing approximately % of our common stock. In particular, ALS Invest 1 B.V., or ALS Invest, will own approximately % of our common stock following this offering, Morningside Venture Investments Limited, or Morningside, will own approximately % of our common stock following this offering and Viking Global Opportunities Illiquid Investments Sub-Master LP, or Viking, will own approximately % of our common stock following this offering. As a result, if ALS Invest, Morningside and Viking, along with stockholders who own more than 5% of our outstanding common stock after this offering were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership control may:

- delay, defer or prevent a change in control;
- entrench our management or the board of directors; or
- impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the price at which shares are being sold in this offering and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors or they may want us to pursue strategies that deviate from the interests of other stockholders.

Delaware law and provisions in our amended and restated certificate of incorporation and amended and restated bylaws that will be in effect at the closing of this offering could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Provisions of our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective immediately prior to and upon the closing of this offering, respectively, may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our common stock.

Among other things, our amended and restated certificate of incorporation and amended and restated bylaws will:

- permit our board of directors to issue up to shares of preferred stock, with any rights, preferences and privileges as they may designate (including the right to approve an acquisition or other change in our control);
- provide that the authorized number of directors may be changed only by resolution of the board of directors;
- provide that our board of directors or any individual director may only be removed with cause and the affirmative vote of the holders of at least 66-2/3% of the voting power of all of our then-outstanding common stock;
- provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- divide our board of directors into three classes;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner and also specify requirements as to the form and content of a stockholder's notice;
- do not provide for cumulative voting rights (therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose);
- provide that special meetings of our stockholders may be called only by the chair of our board of directors, our Chief Executive Officer or by the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors; and
- provide that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for the following types of actions or proceedings under state, statutory and common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (iii) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws; (iv) any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws; (v) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers or other employees governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants; provided these provisions of our amended and restated certificate of incorporation and amended and restated bylaws will not apply to suits brought to enforce a duty or liability created by the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction; and provided that, unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act of 1933, as amended (Securities Act).

The amendment of any of these provisions, with the exception of the ability of our board of directors to issue shares of preferred stock and designate any rights, preferences and privileges thereto, would require approval by the holders of at least 66-2/3% of our then-outstanding common stock.

In addition, as a Delaware corporation, we are subject to Section 203 of the Delaware General Corporation Law. These provisions may prohibit large stockholders, in particular those owning 15% or more of our outstanding voting stock, from merging or combining with us for a certain period of time. A Delaware corporation may opt out of this provision by express provision in its original certificate of incorporation or by amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision.

These and other provisions in our amended and restated certificate of incorporation, amended and restated bylaws and Delaware law could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by our then-current board of directors, including delay or impede a merger, tender offer or proxy contest involving our company. The existence of these provisions could negatively affect the price of our common stock and limit opportunities for you to realize value in a corporate transaction.

For information regarding these and other provisions, see “Description of Capital Stock.”

As a result of becoming a public company, we will be obligated to develop and maintain proper and effective internal controls over financial reporting and any failure to maintain the adequacy of these internal controls may adversely affect investor confidence in our company and, as a result, the value of our common shares.

We will be required, pursuant to Section 404 of the Sarbanes Oxley Act, or Section 404, to furnish a report by management on, among other things, the effectiveness of our internal controls over financial reporting for the fiscal year ending December 31, 2023. This assessment will need to include disclosure of any material weaknesses identified by our management in our internal controls over financial reporting. Our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting until our first annual report required to be filed with the SEC following the date we are no longer an emerging growth company, as defined in the JOBS Act, and are not a smaller reporting company with less than $100 million in annual revenue. At such time as we are required to obtain auditor attestation, if we then have a material weakness, we would receive an adverse opinion regarding our internal control over financial reporting from our independent registered public accounting firm. We will be required to disclose significant changes made in our internal controls procedures on a quarterly basis.

We are beginning the costly and challenging process of enhancing our financial reporting systems and processes as necessary to allow for the operation of effective internal controls over financial reporting to comply with the requirements of Section 404. We may not be able to complete our assessment, testing and any required remediation of internal controls over financial reporting in a timely fashion. Our compliance with Section 404 will require that we incur substantial legal, accounting and other compliance expense and expend significant management efforts. We currently do not have an internal audit group. We will need to hire additional accounting and finance personnel and consultants with appropriate public company experience and technical accounting knowledge to develop and maintain the internal controls over financial reporting necessary to comply with Section 404.

We have identified existing material weaknesses in our internal controls over financial reporting. If during the evaluation and testing of our internal controls over financial reporting, we identify one or

more additional material weaknesses in future periods, we will be unable to assert that our internal controls over financial reporting are effective. We cannot assure you that there will not be additional material weaknesses in our internal controls over financial reporting in the future. Any failure to maintain effective internal controls over financial reporting could severely inhibit our ability to accurately report our financial condition or results of operations. If we are unable to conclude that our internal controls over financial reporting are effective, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common shares could decline, and we could be subject to sanctions or investigations by the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal controls over financial reporting, or to implement or maintain other effective control systems required of public companies, could also negatively impact our ability to access to the capital markets.

In addition, effective disclosure controls and procedures enable us to make timely and accurate disclosure of financial and non-financial information that we are required to disclose. As a public company, if our disclosure controls and procedures are ineffective, we may be unable to report our financial results or make other disclosures accurately and on a timely basis, which could cause our reported financial results or other disclosures to be materially misstated and result in the loss of investor confidence and cause the market price of our common shares to decline.

If we fail to remediate our material weaknesses in internal controls over financial reporting and to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations, meet our reporting obligations or prevent fraud, and investor confidence in our company and the market price of our common stock may be materially and adversely affected.

Prior to the completion of this offering, we had limited accounting personnel, IT personnel and other resources with which to address internal controls over financial reporting. In connection with the audits of our consolidated financial statements as of and for the years ended December 31, 2019 and 2020, we identified two material weaknesses in our internal controls over financial reporting. As defined in the standards established by the U.S. Public Company Accounting Oversight Board, or PCAOB, a “material weakness” is a deficiency, or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the annual or interim financial statements will not be prevented or detected on a timely basis.

Deficiencies in our internal controls over financial reporting that were considered to be a material weakness as of December 31, 2019 were related to the lack of a sufficiently precise review over both valuations prepared by our third-party valuation experts as well as the completeness of operating expenses. We remediated the material weakness related to the review of valuation reports by adding a precise review control that was performed by our accounting personnel with the appropriate technical expertise to review valuation reports. In addition, we have hired an accounting executive with the requisite knowledge in the application of U.S. GAAP and SEC reporting who will be collaborating and reviewing valuation reports prepared by our third-party valuation experts. For the deficiency related to the completeness of operating expenses, we continue to take measures to remediate the deficiency by updating our internal controls to include additional staffing as well as augmenting our controls addressing the completeness of operating expenses, as outlined below.

Further deficiencies in our internal controls over financial reporting that have been considered to be a material weakness as of December 31, 2020 relate to deficiencies in the design of controls over the expenditures process. Specifically, our information technology controls related to the expenditures cycle were not designed to post invoices approved in the correct period, and our controls over the review of the completeness of operating expenses as it related to our close cycle were not

appropriately designed, as we lacked sufficient personnel in our Finance and IT organizations to review and provide reasonable assurance that transactions were being recorded timely and completely. We are in the process of implementing changes to our internal controls over financial reporting to remediate this material weakness that has been identified and these changes include hiring a sufficient number of accounting and IT personnel to focus on our information technology systems and to adequately manage the monthly close process. However, we cannot assure you that these measures will fully address the material weaknesses and deficiencies in our internal control over financial reporting such that we may conclude that they have been fully remediated.

Upon completion of this offering, we will become subject to the Sarbanes-Oxley Act. Section 404 will require that we include a report from management on the effectiveness of our internal control over financial reporting in our annual report on Form 10-K beginning with our annual report in our second annual report on Form 10-K after becoming a public company. In addition, after we become a public company, our reporting obligations may place a significant strain on our management, operational and financial resources and systems for the foreseeable future. We may be unable to timely complete our evaluation testing and any required remediation.

During the course of documenting and testing our internal control procedures, in order to satisfy the requirements of Section 404, we may identify other weaknesses and deficiencies in our internal control over financial reporting. If we fail to maintain the adequacy of our internal control over financial reporting, as these standards are modified, supplemented or amended from time to time, we may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting in accordance with Section 404.

Generally speaking, if we fail to achieve and maintain an effective internal control environment, it could result in material misstatements in our financial statements and could also impair our ability to comply with applicable financial reporting requirements and related regulatory filings on a timely basis. As a result, our businesses, financial condition, results of operations and prospects, as well as the trading price of our common stock, may be materially and adversely affected. Additionally, ineffective internal control over financial reporting could expose us to increased risk of fraud or misuse of corporate assets and subject us to potential delisting from the stock exchange on which we list, regulatory investigations and civil or criminal sanctions. We may also be required to restate our financial statements from prior periods.

Our amended and restated bylaws will provide that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our amended and restated bylaws will provide that, to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants, the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action or proceeding asserting a breach of fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders;
- any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees arising out of or pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or bylaws;

- any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws;
- any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and
- any action asserting a claim against us or any of our directors, officers or other employees that is governed by the internal affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that we will need significant additional capital in the future to continue our planned operations, including conducting clinical trials, commercialization efforts if we are able to obtain marketing approval of any of AMX0035 or any future product candidates, research and development activities, and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock, including shares of common stock sold in this offering.

Pursuant to our 2022 Plan, our management is authorized to grant stock options to our employees, directors and consultants. Additionally, the number of shares of our common stock reserved for issuance under our 2022 Plan will automatically increase on January 1 of each year, beginning on January 1, 2022 and continuing through and including January 1, 2031, by % of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. In addition, pursuant to our

ESPP, the number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, beginning on January 1, 2022 (through January 1, 2031), by the lesser of (i) % of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of the automatic increase, and (ii) shares; provided that before the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

General Risk Factors

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting, and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Exchange Act, which will require, among other things, that we file with the SEC annual, quarterly, and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and the Nasdaq Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act (Dodd-Frank Act) was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Emerging growth companies and smaller reporting companies are exempted from certain of these requirements, but we may be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition, and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

Cyber-attacks or other failures in our telecommunications or information technology systems, or those of our collaborators, CROs, third-party logistics providers, distributors or other contractors or consultants, could result in information theft, data corruption and significant disruption of our business operations.

We, our collaborators, our CROs, third-party logistics providers, distributors and other contractors and consultants utilize information technology, or IT, systems and networks to process, transmit and

store electronic information in connection with our business activities. As use of digital technologies has increased, cyber incidents, including third parties gaining access to employee accounts using stolen or inferred credentials, computer malware, viruses, spamming or other means, and deliberate attacks and attempts to gain unauthorized access to computer systems and networks, have increased in frequency and sophistication. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient. These threats pose a risk to the security of our, our collaborators', our CROs', third-party logistics providers', distributors' and other contractors' and consultants' systems and networks, and the confidentiality, availability and integrity of our data. There can be no assurance that we will be successful in preventing cyber-attacks or successfully mitigating their effects. Similarly, there can be no assurance that our collaborators, CROs, third-party logistics providers, distributors and other contractors and consultants will be successful in protecting our clinical and other data that is stored on their systems. Any cyber-attack, data breach or destruction or loss of data could result in a violation of applicable U.S. and international privacy, data protection and other laws, and subject us to litigation and governmental investigations and proceedings by federal, state and local regulatory entities in the United States and by international regulatory entities, resulting in exposure to material civil and/or criminal liability. Further, our general liability insurance and corporate risk program may not cover all potential claims to which we are exposed and may not be adequate to indemnify us for all liability that maybe imposed; and could have a material adverse effect on our business and prospects. For example, the loss of clinical trial data from completed, ongoing or future clinical trials for AMX0035 or any of our future product candidates could result in delays in our development and regulatory approval efforts and significantly increase our costs to recover or reproduce the data. In addition, we may suffer reputational harm or face litigation or adverse regulatory action as a result of cyber-attacks or other data security breaches and may incur significant additional expense to implement further data protection measures.

Our ability to use net operating losses and research and development credits to offset future taxable income may be subject to certain limitations.

As of December 31, 2020, we had U.S. federal and state net operating loss carryforwards of $46.8 million and $44.9 million, respectively, some of which begin to expire in 2034. As of December 31, 2020, we also had U.S. federal and state research and development tax credit carryforwards of $1.6 million and $0.7 million, respectively, which begin to expire in 2029. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future taxable income or tax liabilities, respectively. U.S. federal and certain state net operating losses generated in taxable years beginning after December 31, 2017 are not subject to expiration. Federal net operating losses generally may not be carried back to prior taxable years except that, under the Coronavirus Aid, Relief and Economic Security Act, federal net operating losses generated in 2018, 2019 and 2020 may be carried back to each of the five taxable years preceding the taxable year in which the loss arises. Additionally, for taxable years beginning after December 31, 2020, the deductibility of federal net operating losses generated in taxable years beginning after December 31, 2017 is limited to 80% of our taxable income in such taxable year.

In general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change net operating loss carryforwards or tax credits, or NOLs or credits, to offset future taxable income. For these purposes, an ownership change generally occurs where the aggregate stock ownership of one or more stockholders or groups of stockholders who owns at least five percent of a corporation's stock increases its ownership by more than 50 percentage points over its lowest ownership percentage within a specified testing period. Our existing federal and state NOLs and our existing research and development credits may be subject to limitations arising from previous ownership changes and, if we undergo an ownership change, our ability to utilize NOLs or credits could be further limited by Sections 382 and 383 of the Code. We have not yet completed a

Section 382 analysis. In addition, this offering or future changes in our stock ownership, many of which are outside of our control, could result in an ownership change under Sections 382 and 383 of the Code. Our NOLs or credits may also be impaired under state law. Accordingly, we may not be able to utilize a material portion of our NOLs or credits. Furthermore, our ability to utilize our NOLs or credits is conditioned upon our attaining profitability and generating U.S. federal and state taxable income. As described above, we have incurred significant net losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future; and therefore, we do not know whether or when we will generate the U.S. federal or state taxable income necessary to utilize our NOLs or credits that are subject to limitation by Sections 382 and 383 of the Code.

We may not be entitled to forgiveness of our recently received PPP Loan, and our application for the PPP Loan could in the future be determined to have been impermissible or could result in damage to our reputation.

In April 2020, we received proceeds of $0.3 million from a loan, or the PPP Loan, under the Paycheck Protection Program, or the PPP, of the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, a portion of which may be forgiven, which we have used to retain employees, maintain payroll and make lease and utility payments. The PPP Loan had a maturity date of April 19, 2022 and an annual interest rate of 1.0%. Payments of principal and interest on the PPP Loan were originally deferred for the first six months of the term. Thereafter, we were required to pay the lender equal monthly payments of principal and interest.

Under the PPP, we applied for and were granted forgiveness for the entirety of the PPP Loan. The amount of loan proceeds eligible for forgiveness was originally based on a formula that takes into account a number of factors, including the amount of loan proceeds used by us during the eight-week period after the loan origination for certain purposes, including payroll costs, interest on certain mortgage obligations, rent payments on certain leases, and certain qualified utility payments, provided that at least 75% of the loan amount was used for eligible payroll costs. Subject to the other requirements and limitations on loan forgiveness, only loan proceeds spent on payroll and other eligible costs during the covered eight-week period qualified for forgiveness.

In order to apply for the PPP Loan, we were required to certify, among other things, that the current economic uncertainty made the PPP Loan request necessary to support our ongoing operations. We made this certification in good faith after analyzing, among other things, our financial situation and access to alternative forms of capital, and believe that we satisfied all eligibility criteria for the PPP Loan, and that our receipt of the PPP Loan is consistent with the broad objectives of the PPP. The certification described above does not contain any objective criteria and is subject to interpretation. If, despite our good-faith belief that given our Company's circumstances we satisfied all eligible requirements for the PPP Loan, we are later determined to have violated any of the laws or governmental regulations that apply to us in connection with the PPP Loan, such as the FCA, or it is otherwise determined that we were ineligible to receive the PPP Loan, we may be subject to penalties, including significant civil, criminal and administrative penalties and could be required to repay the PPP Loan in its entirety. In addition, receipt of a PPP Loan may result in adverse publicity and damage to reputation, and a review or audit by the U.S. Small Business Administration or other government entity or claims under the FCA could consume significant financial and management resources. Notwithstanding the forgiveness of the PPP Loan, on October 7, 2021, we repaid the PPP Loan in full.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because

biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

Our failure to meet Nasdaq's continued listing requirements could result in a delisting of our common stock.

If, after listing, we fail to satisfy the continued listing requirements of the Nasdaq Global Market, such as the corporate governance requirements or the minimum closing bid price requirement, may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase our common stock when you wish to do so. In the event of a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping below the minimum bid price requirement or prevent future non-compliance with the listing requirements of the Nasdaq Global Market.

If securities analysts publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock depends in part on the research and reports that industry or securities analysts publish about us or our business. If one or more of the analysts who cover us issues an adverse opinion about our company, our stock price would likely decline. If one or more of these analysts ceases research coverage of us or fails to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, among other things, statements about:

- our use of the net proceeds from this offering;
- our ability to obtain and maintain regulatory approval of AMX0035 and any future product candidates;
- our ability to successfully commercialize and market AMX0035 and any future product candidates, if approved;
- our ability to contract with third-party suppliers, manufacturers and other service providers and their ability to perform adequately;
- the potential market size, opportunity and growth potential for AMX0035 and any future product candidates, if approved;
- our ability to build our own sales and marketing capabilities, or seek collaborative partners, to commercialize AMX0035 and any future product candidates, if approved;
- our ability to obtain funding for our operations;
- the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development activities;
- our ability to retain the continued service of our key professionals and to identify, hire and retain additional qualified professionals;
- our ability to advance AMX0035 and any future product candidates into, and successfully complete, clinical trials;
- our ability to recruit and enroll suitable patients in our clinical trials;
- the timing or likelihood of the accomplishment of various scientific, clinical, regulatory filings and approvals and other product development objectives;
- the pricing and reimbursement of our product candidates, if approved;
- the degree of market acceptance AMX0035 and any future product candidates by physicians, patients, third-party payors and others in the medical community;
- the rate and degree of market acceptance of AMX0035 and any future product candidates, if approved;
- the implementation of our business model, strategic plans for our business, product candidates and technology;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- developments relating to our competitors and our industry;

- the accuracy of our estimates regarding expenses, capital requirements and needs for additional financing;
- our financial performance; and
- other risks and uncertainties, including those listed under the section titled “Risk Factors.”

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly in the “Risk Factors” section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments that we may make or enter into.

You should read this prospectus and the documents that we have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

USE OF PROCEEDS

We estimate that the net proceeds to us from our issuance and sale of shares of our common stock in this offering will be approximately $ million, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise in full their option to purchase additional shares, we estimate that the net proceeds from this offering will be approximately $ million, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

A $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease our net proceeds from this offering by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions. An increase or decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase or decrease our net proceeds from this offering by approximately $ million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions.

We currently estimate that we will use the net proceeds from this offering, together with our existing cash, cash equivalents, and short-term investments, as follows:

- approximately $ million to fund the regulatory approval process and pre-commercial launch, production of and, if approved, commercial launch activities for AMX0035 for the treatment of ALS;
- approximately $ million to fund the completion of our ongoing Phase 3 PHOENIX clinical trial for the treatment of amyotrophic lateral sclerosis, or ALS;
- approximately $ million to fund the development and expansion of our pipeline to address other neurodegenerative indications, and for formulations and derivatives of AMX0035; and
- the remainder for working capital and other general corporate activities, which may include funding for the costs of operating as a public company.

This expected use of the net proceeds from this offering along with our existing cash, cash equivalents, and short-term investments represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. For example, we may use a portion of the net proceeds for the acquisition of businesses or technologies to continue to build our pipeline, our research and development capabilities and our intellectual property position, although we currently have no agreements, commitments or understandings with respect to any such transaction. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development and our sales and marketing and commercialization efforts, demand for our products, if approved, our operating costs, the status of and results from clinical trials, any collaborations that we may enter into with third parties for our product candidates and any unforeseen cash needs. Moreover, our estimates of the costs to fund our trials are based on the current designs of the trials. If we were to modify the design of any of these trials, for instance, to increase the number of patients in the trials, our costs to fund the trials could increase. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

Based on our current plans, we believe that our existing cash, cash equivalents and short-term investments, together with the net proceeds from this offering, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least twelve months after the registration statement of which this prospectus forms a part becomes effective. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. We do not have any committed external source of funds.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

DIVIDEND POLICY

We have never declared nor paid cash dividends on our common stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. We do not intend to pay cash dividends in respect of our common stock in the foreseeable future.

CAPITALIZATION

The following table sets forth our cash, cash equivalents, and short-term investments and our capitalization as of September 30, 2021:

- on an actual basis;
- on a pro forma basis to (i) give effect to the automatic conversion of all outstanding shares of our preferred stock into an aggregate of shares of common stock upon the closing of this offering, and (ii) the filing and effectiveness of our restated certificate of incorporation upon the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of shares of our common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Our capitalization following the closing of this offering will depend on the actual initial public offering price and other terms of this offering determined at pricing. Cash, cash equivalents, and short-term investments are not components of our total capitalization. You should read this table together with our financial statements and the related notes appearing at the end of this prospectus and the section of this prospectus titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations."

	As of September 30, 2021		
	Actual	Pro Forma	Pro Forma As Adjusted
	(in thousands, except share and per share data)		
Cash, cash equivalents, and short-term investments	$ 125,702	$ 125,702	$
Series A redeemable convertible preferred stock, $0.0001 par value; 6,289,609 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	7,675		
Series B redeemable convertible preferred stock, $0.0001 par value; 15,100,000 shares authorized, 14,496,835 issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	64,387		
Series C-1 redeemable convertible preferred stock, $0.0001 par value; 13,150,430 shares authorized, issued and outstanding, actual, no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	134,791		
Series C-2 redeemable convertible preferred stock, $0.0001 par value; 3,170,585 shares authorized, issued and outstanding, actual, no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	32,498		
Stockholders’ (deficit) equity:			
Common stock, $0.0001 par value; 56,500,000 shares authorized, 6,799,157 shares issued and outstanding, actual; shares authorized, 46,273,487 shares issued and outstanding, pro forma; shares authorized, shares issued and outstanding, pro forma as adjusted	1	5	
Additional paid-in capital	3,431	242,778	
Accumulated deficit	(127,501)	(127,501)	
Accumulated other comprehensive loss	(1)	(1)	
Total stockholders’ (deficit) equity	(124,070)	115,281	
Total capitalization	$ 115,281	$ 115,281	$

A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash, cash equivalents, and short-term investments, additional paid-in capital, total stockholders' equity (deficit) and total capitalization by $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash, cash equivalents, and short-term investments, additional paid-in capital, total stockholders' equity (deficit) and total capitalization by $ million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions.

The table above excludes:

- 4,195,341 shares of our common stock issuable upon the exercise of stock options outstanding as of September 30, 2021, at a weighted average exercise price of $4.31 per share;
- 2,809,492 shares of our common stock available for future issuance as of September 30, 2021 under our 2015 Plan which will cease to be available for issuance at the time that our 2022 Stock Option and Incentive Plan, or the 2022 Plan, becomes effective;
- shares of our common stock that will become available for future issuance under our 2022 Stock Option and Incentive Plan, which will become effective in connection with the completion of this offering, as well as any future increases, including annual automatic evergreen increases, in the number of shares of common stock reserved for issuance thereunder in accordance with the terms of such plan; and
- shares of our common stock that will become available for future issuance under our 2022 Employee Stock Purchase Plan, which will become effective in connection with the completion of this offering.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

Our historical net tangible book value (deficit) as of September 30, 2021 was $(124.1) million, or $(18.25) per share of our common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and redeemable convertible preferred stock, which is not included within stockholders' equity (deficit). Historical net tangible book value (deficit) per share represents our historical net tangible book value (deficit) divided by the 46,273,487 shares of our common stock outstanding as of September 30, 2021.

Our pro forma net tangible book value as of September 30, 2021 was $115.3 million, or $2.49 per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of shares of our common stock upon the closing of this offering. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of September 30, 2021, after giving effect to the foregoing adjustments and the automatic conversion of all outstanding shares of our preferred stock into common stock upon the closing of this offering.

After giving further effect to our issuance and sale of shares of our common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of September 30, 2021 would have been $ million, or $ per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $ to existing stockholders and immediate dilution of $ in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Assumed initial public offering price per share		$
Historical net tangible book value (deficit) per share as of September 30, 2021	$(18.25)	
Increase per share attributable to the pro forma adjustments described above	20.74	
Pro forma net tangible book value (deficit) per share	2.49	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing shares in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Dilution per share to new investors purchasing shares in this offering		$

A $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease our pro forma as adjusted net tangible book value by $ million, our pro forma as adjusted net tangible book value per share after this offering by $ and dilution per share to

new investors purchasing shares in this offering by $, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions. An increase of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase the pro forma as adjusted net tangible book value per share after this offering by $ and decrease the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price and after deducting estimated underwriting discounts and commissions. A decrease of shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would decrease the pro forma as adjusted net tangible book value per share after this offering by $ and increase the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price and after deducting estimated underwriting discounts and commissions.

If the underwriters exercise their option to purchase additional shares in full, our pro forma as adjusted net tangible book value per share after this offering would be $ per share, representing an immediate increase in pro forma as adjusted net tangible book value per share of $ to existing stockholders and immediate dilution in pro forma as adjusted net tangible book value per share of $ to new investors purchasing common stock in this offering, assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If any shares are issued upon exercise of outstanding options or the outstanding warrant, you will experience further dilution.

The following table summarizes, on the pro forma as adjusted basis described above, the differences between the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid by existing stockholders and by new investors purchasing shares of common stock in this offering. The calculation below is based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders		%	$	%	$
New investors					$
Total		100.0%		100.0%	

A $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the total consideration paid by new investors by $ million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by percentage points, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same. An increase or decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase or decrease the total consideration paid by new investors by $ million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by percentage points, assuming no change in the assumed initial public offering price.

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to % of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors participating in the offering would be increased to % of the total number of shares of our common stock outstanding after this offering.

The number of shares purchased from us by existing stockholders is based on 46,273,487 shares of our common stock outstanding as of September 30, 2021 after giving effect to the automatic conversion of all outstanding shares of our preferred stock into an aggregate of shares of common stock upon the closing of this offering, and excludes:

- 4,195,341 shares of our common stock issuable upon the exercise of stock options outstanding as of September 30, 2021, at a weighted average exercise price of $4.31 per share;
- 2,809,492 shares of our common stock available for future issuance as of September 30, 2021 under our 2015 Plan which will cease to be available for issuance at the time that our 2022 Stock Option and Incentive Plan, or the 2022 Plan, becomes effective;
- shares of our common stock that will become available for future issuance under our 2022 Stock Option and Incentive Plan, which will become effective in connection with the completion of this offering, as well as any future increases, including annual automatic evergreen increases, in the number of shares of common stock reserved for issuance thereunder in accordance with the terms of such plan; and
- shares of our common stock that will become available for future issuance under our 2022 Employee Stock Purchase Plan, which will become effective in connection with the completion of this offering.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes included at the end of this prospectus. This discussion and other parts of this prospectus contain forward-looking statements that involve risks and uncertainties, such as statements of our plans, objectives, expectations and intentions. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

Our mission is to develop therapies that change the treatment paradigm for amyotrophic lateral sclerosis, or ALS, and a broad range of neurodegenerative diseases by keeping neurons alive. Unlike most other cells in the body that regularly die and are replaced as part of healthy function, mature neurons are normally resistant to cell death and generally cannot regenerate. We are pursuing commercialization of our product candidate, AMX0035, which we believe is the first drug candidate to show both a functional and survival benefit in a large-scale clinical trial of patients with ALS. We submitted a New Drug Submission, or NDS, in Canada in the second quarter of 2021 for AMX0035 for the treatment of ALS and a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, in the fourth quarter of 2021. We also intend to submit a Marketing Authorization Application, or MAA, in Europe in the first quarter of 2022. In November 2021, we announced that we had dosed the first participants in our global Phase 3 clinical trial evaluating the safety and efficacy of AMX0035 for the treatment of ALS, known as the PHOENIX trial. The results of our Phase 2 clinical trial of AMX0035, known as the CENTAUR trial, were published in September 2020 in the *New England Journal of Medicine* and in October 2020 in the *Journal of Muscle and Nerve* and demonstrated functional and survival benefits for ALS patients. We believe AMX0035 has the potential to be a foundational therapy, meaning that it could be used alone or in conjunction with other therapies to change the treatment paradigm across a broad range of neurodegenerative diseases.

AMX0035 is a dual UPR-Bax apoptosis inhibitor composed of sodium phenylbutyrate, or PB, and TURSO (also known as tauroursodeoxycholic acid, or TUDCA). Through the resolution of the unfolded protein response, or UPR, and by inhibiting translocation of the Bcl-2 Associated X-protein, or Bax, to the outer mitochondrial membrane, we have shown in multiple models that AMX0035 can keep neurons alive under a variety of different conditions and stresses. We are pursuing ALS as our first indication as it is a disease of rapid and profound neurodegeneration, and we are focused on the development and potential commercialization of AMX0035 for ALS globally.

We were incorporated under the laws of the State of Delaware on January 10, 2014. In October 2020 and August 2021, we created wholly owned subsidiaries, Amylyx Pharmaceuticals Canada, Inc., or Amylyx Canada, in Calgary, Canada and Amylyx Pharmaceuticals EMEA B.V, or Amylyx EMEA, in Amsterdam, Netherlands. Amylyx EMEA did not have operations as of September 30, 2021. Since inception, we have devoted substantially all of our efforts to research and development activities, including recruiting management and technical staff, raising capital, producing materials for non-clinical and clinical studies, and building infrastructure to support such activities. Our expenses have primarily been for research and development and related general and administrative costs. We have generated revenues through five grants from ALS Association, ALS Finding a Cure Foundation, Cure Alzheimer's Fund, Alzheimer's Drug Discovery Foundation and Alzheimer's Association, or the Grantors.

Since inception, we have also financed our operations through the issuance of redeemable convertible preferred stock, convertible notes and, to a lesser extent, a government loan. From August 2016 to November 2017, we issued and sold Series A preferred stock for an aggregate purchase price

of approximately $7.7 million. In July 2017, we received $2.3 million from the issuance of convertible promissory notes, or the 2017 Notes. In November 2018, we received $13.0 million from the issuance of convertible promissory notes, or the 2018 Notes. In December 2019, we received $0.6 million from the issuance of convertible promissory notes, or the 2019 Notes. In January, February, and April 2020, we received $15.4 million in aggregate from the issuance of convertible promissory notes, or the 2020 Notes. The 2017 Notes, 2018 Notes, 2019 Notes and 2020 Notes, or the Old Notes, were to mature on December 31, 2021. We also received $0.3 million of net proceeds in April 2020 pursuant to a loan under the Paycheck Protection Program, or the PPP, of the Coronavirus Aid, Relief, and Economic Security (CARES) Act, the PPP Loan, which we repaid in full in October 2021. In June 2020, we issued and sold shares of Series B preferred stock for an aggregate purchase price of approximately $30.0 million. The Old Notes automatically converted into shares of Series B preferred stock pursuant to their original terms in June 2020 in connection with our Series B financing. From December 2020 to February 2021, we received $27.3 million from the issuance of convertible promissory notes, or the 2021 Notes, of which $1.2 million was received in December 2020 and $26.1 million was received in January and February 2021. In July 2021, we issued and sold shares of Series C-1 preferred stock, or the Series C-1 preferred stock, for an aggregate purchase price of approximately $135.0 million. The 2021 Notes automatically converted into shares of Series C-2 preferred stock pursuant to their original terms in July 2021 in connection with our sale of Series C-1 preferred stock.

We have incurred operating losses since inception, including a net loss of $13.7 million and $42.3 million for the years ended December 31, 2019 and 2020, respectively. Our net losses were $33.7 million and $59.6 million for the nine months ended September 30, 2020 and 2021, respectively. As of September 30, 2021, we had an accumulated deficit of $127.5 million. These losses have resulted primarily from costs incurred in connection with research and development activities and general and administrative costs associated with our operations. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future as we advance AMX0035 and any future product candidates through preclinical and clinical development, hire additional clinical, scientific, management and administrative personnel, seek regulatory approval and pursue commercialization of any approved product candidates. To date, we have primarily developed AMX0035 internally, with assistance from our network of contract research organizations, or CROs, and other advisors. This has resulted in increased research and development spending but has enabled us to manage AMX0035 efficiently through the development and manufacturing process.

Following the closing of this offering, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company. As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through the sale of equity, debt financings or other capital sources, including potential collaborations with other companies, royalty financings, or other strategic transactions. Our inability to raise capital as and when needed could have a negative impact on our financial condition and ability to pursue our business strategies. There can be no assurances, however, that the current operating plan will be achieved or that additional funding will be available on terms acceptable to us, or at all.

As of September 30, 2021, we had cash, cash equivalents and short-term investments of $125.7 million. We believe that our existing cash, cash equivalents and short-term investments, together with the net proceeds from this offering, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least twelve months after the registration statement of which this prospectus forms a part becomes effective. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See “—Liquidity and Capital Resources—Funding Requirements” below.

Impact of COVID-19

The development of AMX0035 and any future product candidates could be disrupted and materially adversely affected in the future by a pandemic, epidemic or outbreak of an infectious disease, such as the ongoing COVID-19 pandemic. The spread of COVID-19 and identification of new variants of the virus has impacted the global economy and our operations, including requiring us to make certain alterations to our preclinical and clinical trial activities, such as scheduling certain work off-site and performing off-site assessments. In addition, we had to amend our CENTAUR trial protocol to allow for remote visits by patients, instead of patients making site visits and in certain cases we were forced to delay enrollment at certain sites in our Phase 2 clinical trial for AMX0035 in Alzheimer's disease, or AD.

In spite of current vaccination efforts, if the disruption due to the ongoing COVID-19 pandemic continues, our ongoing global Phase 3 PHOENIX clinical trial for AMX0035 for the treatment of ALS could be delayed due to government orders and site policies on account of the pandemic. Additionally, some patients may be unwilling or unable to travel to study sites, enroll in our trials or be unable to comply with clinical trial protocols, which would delay our ability to conduct preclinical studies and clinical trials or release clinical trial results, as well as delay our ability to obtain regulatory approval for and commercialize AMX0035. Furthermore, COVID-19 could continue to affect our employees or the employees of research sites and service providers on whom we rely as well as those of companies with which we do business, including our suppliers, thereby disrupting our business operations. Existing or renewed quarantines and travel restrictions imposed by governments in the jurisdictions in which we and the companies with which we do business operate could materially impact the ability of employees to access preclinical and clinical sites, laboratories, manufacturing sites and offices. We have implemented and continue to follow work-at-home policies and may experience limitations in employee resources. Our continued reliance on personnel working from home may negatively impact productivity, or disrupt, delay or otherwise adversely impact our business.

In April 2020, we borrowed $0.3 million through the PPP Loan. PPP loans were intended to assist companies impacted by the COVID-19 pandemic to fund certain types of expenditures, including payroll costs, rent and utility payments. The PPP Loan was forgiven in March 2021 and notwithstanding the forgiveness of the PPP Loan, we repaid it in full in October 2021. We are still assessing our business plans and the impact the COVID-19 pandemic may have on our ability to advance the testing, development and manufacturing of AMX0035, including as a result of adverse impacts on the research sites, service providers, vendors, or suppliers on whom we rely, or to raise financing to support the development of AMX0035. No assurances can be given that this analysis will enable us to avoid part or all of any impact from the spread of COVID-19 or its consequences, including downturns in business sentiment generally or in our sector in particular. We cannot presently predict the scope and severity of any potential further disruptions, but if we or any of the third parties on whom we rely or with whom we conduct business, were to experience renewed shutdowns or other business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and adversely impacted.

Components of Our Results of Operations

Revenue

Our revenue to date has been comprised of grant revenue, which are amounts earned from performing contracted research and development services. These grants generally require us to meet certain research milestones in order for funds to be provided. To date, we have not generated any revenue from product sales. If our development efforts for AMX0035 or any future product candidates are successful and result in regulatory approval, or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from product sales or payments from such collaboration or license agreements, or a combination of product sales and payments from such agreements.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the research and development of AMX0035. We expense research and development costs as incurred. These expenses include:

- expenses incurred under agreements with CROs, contract manufacturing organizations, or CMOs, as well as investigative sites and consultants that conduct our clinical trials, preclinical studies and other scientific development services;
- manufacturing scale-up expenses and the cost of acquiring and manufacturing preclinical studies, including manufacturing registration and validation batches, as well as clinical trial materials;
- expenses to acquire technologies to be used in research and development;
- employee-related expenses, including salaries, payroll taxes, related benefits and stock-based compensation expense for employees engaged in research and development functions; and
- costs related to compliance with quality and regulatory requirements.

Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered, or the services rendered.

Certain of our indirect research and development expenses are not tracked on an indication-by-indication basis for AMX0035. We do not allocate employee costs and facilities, including depreciation or other indirect costs, to specific indications because these costs are deployed across multiple indications and, as such, are not separately classified. We use internal resources to oversee the research and discovery as well as to manage our preclinical development, process development, manufacturing and clinical development activities. These employees work across multiple indications and, therefore, we do not track their costs by indication.

Research and development activities are central to our business model. Product candidates such as AMX0035 in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials and related product manufacturing expenses. We expect that our research and development expenses will increase substantially in connection with our planned clinical development activities in the near term and in the future. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the clinical development of AMX0035 and any future product candidates. Our clinical development costs may vary significantly based on factors such as:

- per patient trial costs;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of patients that participate in the trials;
- the number of doses that patients receive;

- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up periods;
- the cost and timing of manufacturing our current or future product candidates;
- the phase of development of our current or future product candidates;
- the efficacy and safety profile of our current or future product candidates; and
- the number of product candidates we are developing.

The successful development and commercialization of AMX0035 and any future product candidates is highly uncertain, due to the numerous risks and uncertainties associated with product development and commercialization, including the following:

- the timing and progress of nonclinical and clinical development activities;
- the number and scope of nonclinical and clinical trials for separate indications we decide to pursue;
- raising necessary additional funds;
- the progress of the development efforts of parties with whom we may enter into collaboration arrangements;
- our ability to maintain our current development activities and to establish new ones;
- our ability to establish new licensing or collaboration arrangements;
- the successful initiation and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to Health Canada, the U.S. Food and Drug Administration, or the FDA, the European Medicines Association, or the EMA, or any other comparable foreign regulatory authority;
- the receipt and related terms of regulatory approvals from applicable regulatory authorities;
- the availability of drug substance and drug product for use in production of AMX0035;
- establishing and maintaining agreements with third-party manufacturers for clinical supply for our clinical trials and commercial manufacturing, if AMX0035 is approved;
- our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- our ability to protect our rights in our intellectual property portfolio;
- the commercialization of AMX0035, if and when approved;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement;
- the acceptance of AMX0035, if approved, by patients, the medical community and third-party payors;
- competition with other product; and
- a continued acceptable safety profile of our therapies following approval.

A change in the outcome of any of these variables with respect to the development of AMX0035 or any future product candidates. We may never succeed in obtaining regulatory approval for AMX0035 or any future product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for personnel in executive, finance, sales, marketing, as well as administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters; professional fees for accounting, auditing, tax and administrative consulting services; insurance costs; administrative travel expenses; marketing expenses; rent expense and other operating costs. We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of AMX0035. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance, and director and officer insurance costs as well as investor and public relations expenses associated with being a public company. Additionally, we are pursuing regulatory approval of AMX0035 for the treatment of ALS, initially in Canada, the United States and Europe. As we prepare for a potential approval in each territory, we anticipate an increase in payroll and expense as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of AMX0035.

Other Income (Expense), Net

Interest Expense

Interest expense consists of coupon interests and amortization of derivative discounts associated with our Old Notes. Also, included in interest expense is a contingent beneficial conversion feature recorded upon conversion of our 2017 Notes into shares of Series B redeemable convertible preferred stock and the immediate charge to interest expense for the remaining unamortized debt discount associated with our 2017 Notes upon conversion.

Interest Income

Interest income consists of interest income earned on our cash and cash equivalents, and money market funds.

Other (Expense) Income, Net

Other (expense) income, net consists primarily of (i) extinguishment gain from the conversion of our 2019 Notes and 2020 Notes into Series B redeemable convertible preferred stock in June 2020, (ii) the amortization of premiums and accretion of discounts on our short-term investments, and (iii) income from our short-term investments and (iv) unrealized gain on foreign exchange transactions.

Change in Fair Value of Derivative Liability

Change in fair value of derivative liability is comprised of adjustments to the fair value of embedded derivatives associated with certain redemption features of our Old Notes.

The Old Notes contain redemption features which we determined were embedded derivatives. For the respective Old Notes, we bundled these features together and accounted for the feature as a single, compound embedded derivative at each issuance. The embedded derivative was recorded as a liability and measured at fair value at inception of the Old Notes. The fair value was remeasured at the end of each reporting period and immediately prior to the conversion of the Old Notes. Changes in the estimated fair value during the period were recorded as a component of other income (expense). Subsequent to

June 2020, when the Old Notes converted into shares of our Series B redeemable convertible preferred stock, we no longer have an outstanding embedded derivative liability. Prior to such conversion, the embedded derivative liability was recorded at fair value utilizing an income approach that identified the cash flows using a “with-and-without” valuation methodology. The inputs used to determine the estimated fair value of the derivative instrument were based primarily on the probability of an underlying event triggering the embedded derivative occurring and the timing of such event.

Change in Fair Value of Convertible Notes

Change in fair value of convertible notes is comprised of adjustments to the fair value of our 2021 Notes. As permitted under ASC Topic 825, *Financial Instruments* (ASC 825), we elected the fair value option to account for our 2021 Notes, and as a result, we measured our 2021 Notes at fair value at each financial reporting period and immediately before conversion in July 2021. All changes to the fair value of our 2021 Notes for the nine months ended September 2021 resulted in a loss. Our 2021 Notes converted into shares of Series C-2 redeemable convertible preferred stock concurrently with the issuance of our Series C-1 redeemable convertible preferred stock. Immediately prior to the conversion, we determined the fair value of our 2021 Notes based on the fair value of the Series C-1 redeemable convertible preferred stock and the conversion price at which these notes converted, which was at 85% of the fair value of the Series C-1 redeemable convertible preferred stock.

Income Taxes

The provision for income taxes primarily consists of state minimum taxes in the United States, which do not fluctuate when there is a pre-tax loss. Since our inception, we have incurred significant net losses and anticipate that we will continue to incur significant losses for the foreseeable future.Therefore, we do not know whether or when we will generate the U.S. federal or state taxable income necessary to utilize our Net Operating Losses, or NOLs, or research and development tax credits.

As of December 31, 2019, and 2020, we had federal net operating loss carryforwards of approximately $10.1 million and $46.8 million, respectively, and state net operating loss carryforwards of approximately $9.2 million and $44.9 million, respectively, which are available to reduce future taxable income. Of the $46.8 million federal net operating loss carryforwards, $1.3 million begin to expire in 2034 and the remaining $45.5 million net operating losses carryforward indefinitely. The $44.9 million of Massachusetts net operating loss carryforwards begin to expire in 2034. As of December 31, 2019, and 2020, we also had federal tax credits of $0.7 million and $1.6 million, respectively, and state tax credits of $0.4 million and $0.7 million, respectively. The tax credit carryforwards will expire at various dates beginning in 2029.

There were no provisions for income taxes for the nine months ended September 30, 2020 and 2021 because we have historically incurred operating losses and we maintain a full valuation allowance against our net deferred tax assets.

Results of Operations

Comparison of the Nine Months Ended September 30, 2020 and 2021

The following table summarizes our results of operations for the nine months ended September 30, 2020 and September 30, 2021:

	Nine Months Ended September 30,			
	2020	2021	$ Change	% Change
	(in thousands)			
Grant revenue	$ 300	$ 285	$ (15)	(5.0)%
Operating expenses:				
Research and development	19,581	30,646	11,065	56.5%
General and administrative	11,132	24,012	12,880	115.7%
Total operating expenses	30,713	54,658	23,945	78.0%
Loss from operations	(30,413)	(54,373)	(23,960)	78.8%
Other income (expense), net:				
Interest income	14	6	(8)	(57.1)%
Interest expense	(2,287)	—	2,287	(100.0)%
Change in fair value of derivative liability	(1,270)	—	1,270	(100.0)%
Change in fair value of convertible notes	—	(5,228)	(5,228)	*NM
Other income, net	268	8	(260)	(97.0)%
Total other expense, net	(3,275)	(5,214)	(1,939)	(354.2)%
Net loss	$(33,688)	$(59,587)	$(25,899)	76.9%

* NM - not meaningful

Grant Revenue

Grant revenue for the nine months ended September 30, 2020 was consistent with the grant revenue for the nine months ended September 30, 2021 at $0.3 million. The immaterial decrease in grant revenue was primarily a result of final milestones being achieved during the nine months ended September 30, 2021.

Research and Development Expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2020 and September 30, 2021:

	Nine Months Ended September 30,			
	2020	2021	$ Change	% Change
	(in thousands)			
AMX 00035 - ALS	$11,023	$11,501	$ 478	4.3%
Other indications	4,106	9,755	5,649	137.6%
Payroll and personnel-related	2,161	5,502	3,341	154.6%
Indirect costs	2,291	3,888	1,597	69.7%
	$19,581	$30,646	$11,065	56.5%

Research and development expenses were $19.6 million for the nine months ended September 30, 2020, as compared to $30.6 million for the nine months ended September 30, 2021. The increase of $11.1 million was primarily due to a $5.6 million increase in spend on indications, a

$3.3 million increase in payroll and personnel-related costs, a $1.6 million increase in all other indirect costs, and a $0.5 million increase in AMX0035 for the ALS indication. The increase in spending on other potential indications was primarily due to increased costs for CROs and consultants to support the advancement of our clinical trial activities. The increase in payroll and personnel-related costs was primarily due to increased payroll expense, including stock-based compensation, as a result of an increase in headcount to support our growth. The increase in indirect costs was primarily due to an increase in consulting costs as a result of our growth in 2021 as compared to 2020. The increase in spending on AMX0035 for the ALS indication was a result of increased program activity for the nine months ended September 30, 2021 as compared to the nine months ended September 30, 2020.

General and Administrative Expenses

General and administrative expenses were $11.1 million for the nine months ended September 30, 2020 compared to $24.0 million for the nine months ended September 30, 2021. The increase of $12.9 million was primarily due a $4.4 million increase in payroll and personnel-related costs, a $1.4 million increase in stock-based compensation expense, and a $7.2 million increase in advertising costs and other professional fees, offset by a $0.1 million decrease in public relations fees. The increase in payroll and personnel-related costs was driven by an increase in headcount, which resulted in an increase in bonus expense, 401(k) match, payroll expense and other personnel-related costs. The increase in stock-based compensation expense was primarily due to an increase in fair value of our common stock and an increase in the number of stock options granted to employees resulting from increased headcount. The increase in advertising and other professional fees was due to increased expenditure on advertising, promotion and marketing to support our growth and expansion, and increased expenditures on legal fees, and outside professional services, including accounting, tax and audit fees. The decrease in public relations fees was primarily driven by fewer public relations activities for the nine months ended September 30, 2021 as compared to 2020.

Other Income (Expense), Net

Interest Income

Interest income for the nine months ended September 30, 2020 and 2021 was less than $0.1 million.

Interest Expense

Interest expense was $2.3 million for the nine months ended September 30, 2020 as compared to no interest expense for the nine months ended September 30, 2021. The interest expense was primarily related to the amortization of the derivative discount associated with our 2020 Notes, the recognition of a contingent beneficial conversion feature associated with our 2017 Notes upon the conversion of these notes into Series B redeemable convertible preferred stock in June 2020, and an immediate charge to interest expense for the unamortized derivative discount associated with our 2017 Notes upon conversion of these notes. We recorded no interest expense for the nine months ended September 30, 2021 as we elected to account for our 2021 Notes under the fair value option. Accordingly, all changes to the fair value of our 2021 Notes, inclusive of interest expense are included in change in fair value of convertible notes.

Change in Fair Value of Derivative Liability

The change in fair value of derivative liability was a $1.3 million loss for the nine months ended September 30, 2020 as compared to no change in fair value of derivative liability for the nine months ended September 30, 2021. The change in fair value of derivative liability was related to the issuance

of our 2020 Notes, which included embedded derivatives, and a change in the fair value of our 2019 and 2020 notes resulting from a change in the probability of the settlement scenarios for these notes, including the timing of the conversion of these notes.

Change in Fair Value of Convertible Notes

There was no change in fair value of convertible notes for the nine months ended September 30, 2020 as compared to a $5.2 million recorded for the nine months ended September 30, 2021. There was no change in fair value of convertible notes for the nine months ended September 30, 2021 as we did not elect to account for our Old Notes under the fair value option. The $5.2 million recorded for the nine months ended September 30, 2021 represented a loss and was related to our 2021 Notes, which were measured quarterly at fair value. The change in fair value was primarily due to interest expense for our 2021 Notes at the stated interest rate and the conversion of our 2021 Notes into shares of Series C-2 redeemable convertible preferred stock at 15% discount to the fair value of the Series C-1 redeemable convertible preferred stock issued in July 2021.

Other Income, Net

Other income, net was a gain of $0.3 million for the nine months ended September 30, 2020, compared to a gain of less than $0.1 million for the nine months ended September 31, 2021. The $0.3 million gain in 2020 was related to the extinguishment gain from the conversion of our 2019 Notes and 2020 Notes into 1,058,033 shares of our Series B redeemable convertible preferred stock. The less than $0.1 million of other income for the nine months ended September 30, 2021 was primarily due to unrealized gain on foreign exchange transactions.

Comparison of the Years Ended December 31, 2019 and 2020

The following table summarizes our results of operations for the years ended December 31, 2019 and 2020:

	Year Ended December 31,			
	2019	2020	$ Change	% Change
	(in thousands)			
Grant revenue	$ 1,426	$ 650	$ (776)	(54.4)%
Operating expenses:				
Research and development	11,899	24,594	12,695	106.7%
General and administrative	3,081	15,061	11,980	388.8%
Total operating expenses	14,980	39,655	24,675	164.7%
Loss from operations	(13,554)	(39,005)	(25,451)	187.8%
Other income (expense), net:				
Interest income	176	14	(162)	(92.0)%
Interest expense	(1,276)	(2,288)	(1,012)	79.3%
Change in fair value of derivative liability	939	(1,270)	(2,209)	(235.3)%
Other (expense) income, net	(1)	269	270	*NM
Total other expense, net	(162)	(3,275)	(3,113)	*NM
Net loss and comprehensive loss	$(13,716)	$(42,280)	$(28,564)	208.3%

* NM - not meaningful

Grant Revenue

Grant revenue was $1.4 million for the year ended December 31, 2019, compared to $0.7 million for the year ended December 31, 2020. The decrease of $0.8 million was primarily due to less contracted research and development services being performed during the year ended December 31, 2020 than during the year ended December 31, 2019. We performed less contracted research and development services in 2020 as compared to 2019 as we completed more contracted research and development services in 2019 than in 2020 based on the terms of the grant agreements with our Grantors. Our grant agreements provide estimated timelines over which contracted research and development services would be provided to the Grantors. As less research and development services were scheduled to be provided in 2020, this resulted in the recognition of less revenue during the year ended December 31, 2020 as compared to 2019.

Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2019 and 2020:

	Year Ended December 31,			
	2019	2020	$ Change	% Change
	(in thousands)			
AMX 00035 - ALS	$ 6,958	$12,493	$ 5,535	79.5%
Other indications	3,658	4,356	698	19.1%
Payroll and personnel-related	1,256	3,326	2,070	164.8%
Indirect costs	27	4,419	4,392	*NM
	$11,899	$24,594	$12,695	263.4%

* NM - not meaningful

Research and development expenses were $11.9 million for the year ended December 31, 2019, compared to $24.6 million for the year ended December 31, 2020. During these years, all our research and development expenses were related to the development of and clinical trials of AMX0035. The increase of $12.7 million was primarily due to a $5.5 million increase in spending on AMX0035 for the ALS indication, a $0.7 million increase in spending on the other indications, a $2.1 million increase in payroll and personnel-related costs, and a $4.4 million increase in all other indirect costs. The increases in spending on AMX0035 were primarily as a result of increased program activity in the year ended December 31, 2020 as compared to the year ended December 31, 2019, as we purchased more manufacturing supplies and ran more validation batches in anticipation of commercialization. The increase in payroll-related costs and health insurance was primarily due to increase in the number of employees in our research and development department. Increase in the indirect costs is primarily due to the increase in consulting costs due to increase used of consultants in our research and development department in 2020 as compared to 2019.

General and Administrative Expenses

General and administrative expenses were $3.1 million for the year ended December 31, 2019 compared to $15.1 million for the year ended December 31, 2020. The increase of $12.0 million was primarily due to a $3.0 million increase in commercial expenses, a $5.6 million increase in professional fees, a $3.3 million increase in payroll-related costs and a $0.1 million increase in rent expense. The increase in commercial expenses was primarily due to an increase in spending for brand development, public relations and market research for AMX0035 as we pursue commercialization. The increase in professional fees was primarily due to increased expenditure on legal fees and outside professional

services, including accounting, tax and audit fees. The increase in payroll related costs was primarily due to increase in headcount, related to hiring additional personnel in general and administrative functions to support our growth initiatives. The increase in rent expense was primarily due to the lease of an additional office space in 2020.

Other Income (Expense), Net

Interest Income

Interest income for the years ended December 31, 2019 and 2020 was $0.2 million and less than $0.1 million, respectively. The decrease in interest income was primarily due to a decrease in interest rates during the year ended December 31, 2020, as compared to the year-ended December 31, 2019.

Interest Expense

Interest expense was $1.3 million for the year ended December 31, 2019 compared to $2.3 million for the year ended December 31, 2020. The increase of $1.0 million was primarily due to $1.6 million of interest expense recorded as a result of the amortization of derivative discount associated with our 2020 Notes, the recognition of a contingent beneficial conversion feature associated with our 2017 Notes upon the conversion of these notes into Series B redeemable convertible preferred stock in June 2020, and immediate charge to interest expense for the unamortized derivative discount associated with our 2017 Notes upon conversion of these notes, offset by a $0.6 million decrease in interest expense recorded on our 2017 Notes, 2018 Notes and 2019 Notes as these notes converted into Series B redeemable convertible preferred stock in June 2020. This conversion resulted in our recognition of less interest expense on these notes during the year ended December 31, 2020 as compared to year ended December 31, 2019.

Change in Fair Value of Derivative Liability

The change in fair value of derivative liability resulted in a gain of $0.9 million for the year ended December 31, 2019, compared to a loss of $1.3 million for the year ended December 31, 2020. The change of $2.2 million was primarily due to the issuance of the 2020 Notes, which included embedded derivatives, and a change in the probability related to the settlement scenarios associated with our 2019 Notes and 2020 Notes including the timing of the conversion of these notes.

Other Expense (Income), Net

Other expense, net was a loss of less than $0.1 million for the year ended December 31, 2019, compared to a gain of $0.3 million for the year ended December 31, 2020. The less than $0.1 million of loss in 2019 was related to a loss on foreign currency transaction. The $0.3 million gain in 2020 was related to the extinguishment gain from the conversion of our 2019 Notes and 2020 Notes into 1,058,033 shares of our Series B redeemable convertible preferred stock and represented the difference between the fair value of the Series B redeemable convertible preferred stock of $18.0 million and the carrying value of the 2019 and 2020 Notes including derivative liability of $18.3 million.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have incurred significant operating losses and generated revenues through five grants from the Grantors. We have not yet commercialized any products. To date, we have financed our operations primarily through the sale and issuance of convertible preferred stock,

convertible notes, grant agreements with the Grantors and, to a lesser extent, a government loan. As of September 30, 2021, we had cash, cash equivalents and short-term investments of $125.7 million.

From inception through September 30, 2021, we have raised $234.3 million in aggregate proceeds, net of issuance costs, primarily from the issuance of convertible preferred stock, convertible notes and grant agreements. In July 2021, we issued and sold shares of Series C-1 preferred stock for an aggregate purchase price of approximately $135.0 million. The 2021 Notes automatically converted into shares of Series C-2 preferred stock pursuant to their original terms in July 2021 in connection with our sale of Series C-1 preferred stock. Based on our current operational plans and assumptions, we believe that our existing cash, cash equivalents and short-term investments, together with the net proceeds from this offering, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least twelve months after the registration statement of which this prospectus forms a part becomes effective.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities, manufacturing and clinical trials of AMX0035 and any future product candidates, and prepare for the commercial launch of AMX0035, if approved. In addition, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Our expenses will also increase as we:

- continue our research and development efforts, including our ongoing global Phase 3 PHOENIX trial of AMX0035 for the treatment of ALS;
- pursue commercialization of AMX0035 for the treatment of ALS, initially in Canada, the United States and Europe;
- submit investigational new drug applications, or INDs, of AMX0035 for the treatment of Wolfram syndrome and potentially for other indications;
- conduct preclinical studies and clinical trials for potential future product candidates;
- seek to identify and develop, acquire or in-license additional product candidates;
- experience any delays or encounter any issues with any of the above, including but not limited to failed studies, complex results, safety issues, or other regulatory challenges;
- develop the necessary processes, controls and manufacturing data to obtain marketing approval for AMX0035 or any future product candidates and to support manufacturing on a commercial scale;
- seek regulatory approvals for AMX0035 or any future product candidates that successfully complete clinical trials, if any;
- hire and retain additional personnel, such as non-clinical, clinical, quality assurance, regulatory affairs, manufacturing, distribution, legal, compliance, finance, general and administrative, commercial and scientific personnel;
- develop, maintain, expand and protect our intellectual property portfolio; and
- transition our organization to being a public company.

Following this offering, we will be a publicly traded company and will incur significant legal, accounting and other expenses that we were not required to incur as a private company. In addition,

the Sarbanes-Oxley Act of 2002, as well as rules adopted by the SEC and the Nasdaq Global Market, require public companies to implement specified corporate governance practices that are currently not applicable to us as a private company. Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will first be required to furnish a report by our management on our internal control over financial reporting for the year ending December 31, 2023. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. We expect these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

Based on our current operational plans and assumptions, we expect that the net proceeds from this offering, combined with our current cash, cash equivalents and short-term investments, will be sufficient to fund operations for at least twelve months after the registration statement of which this prospectus forms a part becomes effective. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. As we progress with our development activities and the regulatory review process, we expect to incur significant commercialization expenses related to product manufacturing, pre-commercial activities and commercialization.

Based on our recurring losses, expectation of continuing operating losses and negative cash flows from operations in the foreseeable future, and the need to raise additional capital to finance future operations, we have concluded that there is substantial doubt about our ability to continue as a going concern. The accompanying consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Accordingly, the consolidated financial statements have been prepared on a basis that assumes we will continue as a going concern, and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

Because of the numerous risks and uncertainties associated with research, development and commercialization of product candidates and programs, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical and clinical development for AMX0035 and any future product candidates;
- the costs, timing and outcome of potential future commercialization activities, including manufacturing, marketing, sales and distribution for AMX0035 or any future product candidates for which we receive marketing approval;
- the costs, timing and outcome of regulatory review of AMX0035 and any future product candidates;
- our ability to establish and maintain collaborations and license agreements on favorable terms, if at all;
- our ability to enroll clinical trials in a timely manner and to quickly resolve any delays or clinical holds that may be imposed on our development activities;

- timing delays with respect to preclinical and clinical development of AMX0035 and any future product candidates, including as result of the ongoing COVID-19 pandemic or other pandemics or disruptions;
- the costs of expanding our facilities to accommodate our expected growth in personnel;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire technologies or other assets;
- the sales price and availability of adequate third-party coverage and reimbursement for AMX0035 and any future product candidates, if and when approved; and
- the costs of operating as a public company.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to finance our cash needs through equity offerings, debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, current ownership interests will be diluted. If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Cash Flows

Comparison of the Nine Months Ended September 30, 2020 and 2021

The following table summarizes our sources and uses of cash for the nine months ended September 30, 2020 and September 30, 2021:

	Nine Months Ended September 30,			
	2020	2021	$ Change	% Change
	(in thousands)			
Net cash used in operating activities	$(27,809)	$ (46,555)	$ (18,746)	67.4%
Net cash used in investing activities	—	(49,220)	(49,220)	100.0%
Net cash provided by financing activities	45,645	159,571	113,926	249.6%
Effect of exchange rate changes on cash, cash equivalents and restricted cash	—	4	4	100.0%
Net increase in cash, cash equivalents and restricted cash	$ 17,836	$ 63,800	$ 45,964	257.7%

Operating Activities

During the nine months ended September 30, 2020, operating activities used $27.8 million of cash, primarily resulting from our net loss of $33.7 million, $1.7 million of non-cash interest expense,

$1.3 million of change in fair value of derivative liability, $0.3 million of extinguishment gain from the conversion of our 2019 Notes and 2020 Notes into Series B redeemable convertible preferred stock, $0.1 million of stock-based compensation expense, offset by $3.1 million increase in net cash provided by changes in our operating assets and liabilities. Net cash provided by changes in our operating assets and liabilities was primarily due to favorable changes in working capital.

During the nine months ended September 30, 2021, operating activities used $46.6 million of cash, primarily resulting from our net loss of $59.6 million, $5.2 million of change in fair value of convertible notes, $2.0 million of stock-based compensation expense, $0.1 million of depreciation expense and net amortization of premiums and discounts on investments, offset by $5.7 million increase in net cash provided by changes in our operating assets and liabilities. Net cash provided by changes in our operating assets and liabilities primarily consisted of a $8.1 million increase in accrued expenses and deferred rent due to increased spending for external research and development to support our growth, and a $0.1 million decrease in other assets, offset by a $1.8 million increase in prepaid expenses and other current assets due to increase in sign-on bonuses as a result of an increase in headcount and increase in other receivables related to milestones achieved under the grant agreements for which we were owed by the Grantors, and a $0.7 million net decrease in other working capital accounts.

Investing Activities

There were no cash flows from investing activities during the nine months ended September 30, 2020.

During the nine months ended September 30, 2021, net cash used in investing activities was $49.2 million, resulting from $49.1 million of purchases of short-term investments and $0.2 million of purchases of property and equipment.

Financing Activities

During the nine months ended September 30, 2020, net cash provided by financing activities was $45.6 million. This amount consisted of $30.0 million of net proceeds from the sale of our Series B redeemable convertible preferred stock, $10.6 million of net proceeds from the issuance of the 2020 Notes, $4.8 million of net proceeds from the issuance of the 2020 Notes to related parties, and $0.3 million of proceeds from the PPP loan obtained.

During the nine months ended September 30, 2021, net cash provided by financing activities was $159.7 million. This amount consisted of $134.8 million of net proceeds from the issuance of our Series C-1 redeemable convertible preferred stock, $14.3 million of net proceeds from the issuance of convertible notes to related parties, $11.9 million of net proceeds from the issuance of the convertible notes and $0.2 million of proceeds from exercises of stock options, offset by a $1.5 million payment of deferred offering costs and less than $0.1 million of issuance costs related to the conversion of the convertible notes, which was related to the 2021 Notes.

Comparison of the Years Ended December 31, 2019 and 2020

The following table summarizes our sources and uses of cash for the years ended December 31, 2019 and 2020:

	Year Ended December 31,			
	2019	2020	$ Change	% Change
	(in thousands)			
Net cash used in operating activities	$(10,687)	$(36,697)	$(26,010)	243.4%
Net cash used in investing activities	—	(151)	(151)	100.0%
Net cash provided by financing activities	668	46,823	46,155	*NM
Net (decrease) increase in cash, cash equivalents and restricted cash	$(10,019)	$ 9,975	$ 19,994	(199.6)%

* NM - not meaningful

Operating Activities

During the year ended December 31, 2019, operating activities used $10.7 million of cash, primarily resulting from our net loss of $13.7 million and $0.9 million change in fair value of derivative liability, partially offset by a non-cash interest expense of $0.4 million and net cash provided by changes in our operating assets and liabilities of $3.5 million. Net cash provided by changes in our operating assets and liabilities primarily consisted of a $1.3 million increase in accounts payable due to outstanding invoices to CROs, and other vendors in connection with our increased level of operating activities in 2019, and a $1.3 million increase in accrued expenses and other current liabilities, which was primarily due to increased costs associated with AMX0035.

During the year ended December 31, 2020, operating activities used $36.7 million of cash, primarily resulting from our net loss of $42.3 million and $0.3 million of extinguishment gain from the conversion of our 2019 Notes and 2020 Notes into Series B redeemable convertible preferred stock, partially offset by $1.7 million of non-cash interest expense, $2.7 million of net cash provided by changes in our operating assets and liabilities, $1.3 million of change in fair value of derivative liability, and $0.2 million of non-cash stock compensation expense.

The increase in non-cash interest expense was primarily due to the amortization of the derivative discount associated with the 2020 Notes and the recognition of a contingent beneficial conversion feature associated with our 2017 Notes upon the conversion of these Notes into Series B redeemable convertible preferred stock. Net cash provided by changes in our operating assets and liabilities primarily consisted of a $1.4 million increase in accounts payable, a $1.4 million increase in accrued expenses and other current liabilities and a $0.6 million increase in accrued interest on our Notes, partially offset by a $0.7 million increase in prepaid expenses and other current assets. The increases in accounts payable, accrued expenses and other current liabilities were primarily due to timing of invoicing and cash disbursement to our vendors in connection with our increased level of operating activities in 2020. The increase in prepaid expenses and other current assets was primarily due to subscription to a health data analytics software program used in the research and development of AMX0035 and future product candidates in 2020 and increase in sign-on bonus payments to our employees in our research and development department as a result of an increase in headcount.

Investing Activities

There were no cash flows from investing activities during the year ended December 31, 2019.

During the year ended December 31, 2020, net cash used in investing activities was $0.2 million, driven by purchases of property and equipment.

Financing Activities

During the year ended December 31, 2019, net cash provided by financing activities was $0.7 million, consisting primarily of $0.6 million of net proceeds from the issuance of the 2019 Notes and proceeds from the exercise of stock options of less than $0.1 million.

During the year ended December 31, 2020, net cash provided by financing was $46.8 million, consisting of $30.0 million of net proceeds from the sale of Series B redeemable convertible preferred stock, $15.4 million of net proceeds from the issuance of the 2020 Notes, net of issuance costs, $1.2 million of proceeds received in advance of the issuance of the 2021 Notes and $0.3 million of net proceeds received from the PPP Loan.

In April 2020, we received the PPP Loan from First Republic Bank. Under the terms of the CARES Act and the PPP Loan, all or portion of the principal amount of the PPP Loan is subject to forgiveness so long as, over the 24-week period following our receipt of the proceeds of the PPP Loan, we use those proceeds for payroll costs, rent, utility costs or the maintenance of employee and compensation levels. The PPP Loan was forgiven in March 2021 and notwithstanding the forgiveness of the PPP Loan, we repaid it in full on October 7, 2021.

Contractual Obligations and Commitments

In October 2018, we entered into an operating lease for our office space in Cambridge, Massachusetts, which was to expire on December 31, 2018. In January 2020, we entered into an amendment to extend the lease term of our office space and to lease additional office space, or Expansion Space. Pursuant to the amendment, our lease of both the office space and the Expansion Space will expire in October 2026. The terms of the amendment include an option for a one-time, five-year extension of the lease of the Expansion Space.

We have certain payment obligations under our grant agreements. Under the terms of the respective grant agreements with each Grantor, we will be required to make royalty payments upon future events such as our achievement of commercialization, in connection with the sale of products developed under these agreements and the receipt of cash proceeds resulting from revenue generated from the project for which the grants were used.

For our grant agreements with ALS Association and ALS Finding a Cure, we will be required to pay royalties over time in the amount equal to 150% of the grants received up to a maximum of $2.3 million.

Pursuant to the terms of our grant agreements with Alzheimer’s Drug Discovery Foundation, the Alzheimer’s Association, and Cure Alzheimer’s Fund, we will be required to make royalty payments over time up to the maximum amount of $15.0 million to each Grantor. As the achievement and timing of these future royalty payments are not probable or estimable, such amounts have not been included in our balance sheets as of December 31, 2019, December 31, 2020 and, September 30, 2021.

We enter into contracts in the normal course of business with CROs and other third-party vendors for clinical trials, clinical and commercial supply manufacturing, support for pre-commercial activities, research and development activities and other services and products for our operations. These contracts are generally cancelable upon written notice.

For additional information on our contractual obligation and commitments please see Note 15 — Commitments and Contingencies to our consolidated financial statements and Note 13 to our condensed consolidated financial statements included elsewhere in this prospectus.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with U.S. GAAP. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements appearing at the end of this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments if necessary.

The estimate of accrued research and development expense is dependent, in part, upon the receipt of timely and accurate reporting from CROs, CMOs and other third-party service providers. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with preclinical development activities;
- CROs and investigative sites in connection with preclinical studies and clinical trials; and
- CMOs in connection with drug substance and drug product formulation of preclinical study and clinical trial materials.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CMOs and CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate,

we adjust the accrual or the amount of prepaid expenses accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Valuation of Derivative Liability

In connection with our issuance of the 2017 Notes, 2018 Notes, 2019 Notes, and 2020 Notes, we recognized derivative liabilities associated with the redemption features as they met the requirements for separate accounting as derivatives. The derivative instruments were recorded at fair value at inception and were subject to remeasurement to fair value the end of each reporting period and immediately prior to conversion, with any changes in fair value recognized in the statements of operations and comprehensive loss. The primary inputs for the valuation approach included the probability of achieving various settlement scenarios that provide the lenders the rights or the obligations to receive cash at maturity or a variable number of shares upon the completion of qualified financing, and stock or asset sale. The fair value of the derivative instruments associated with each note was estimated using a two-step approach to valuation, employing a probability-weighted scenario valuation method and then comparing the instrument's value with-and-without the derivative features in order to estimate their combined fair value, using unobservable inputs. In order to estimate the fair value of the 2017, 2018, 2019, and 2020 Notes, we estimated the future payoff in each scenario, discounted them to a present value and then probability weighted them based upon our best estimate of likelihood of each event occurring. In June 2020, in connection with our issuance of the Series B redeemable convertible preferred stock, our 2017, 2018, 2019 and 2020 Notes converted into shares of Series B redeemable convertible preferred stock.

Fair Value Option

As permitted under ASC Topic 825, Financial Instruments (ASC 825), we elected the fair value option to account for our 2021 Notes, which converted into Series C-2 redeemable convertible preferred stock in July 2021. In accordance with ASC 825, we recorded the 2021 Notes at fair value with changes in fair value recorded in the condensed consolidated statement of operations as of September 30, 2021. As a result of applying the fair value option, direct costs and fees related to the 2021 Notes were expensed as incurred and were not deferred. We concluded it was appropriate to apply the fair value option to the 2021 Notes because they are liabilities that are not, in whole or in part, classified as a component of the stockholders' deficit. In addition, the 2021 Notes met other applicable criteria for electing fair value option under ASC 825.

In determining the fair value of the 2021 Notes under the fair value option, we used a scenario-based analysis to incorporate estimates and assumptions concerning our prospects and market indications into a model to estimate the value of the 2021 Notes. The most significant estimates and assumptions used as inputs are those concerning timing, probability of possible scenarios for conversion or settlement of the 2021 Notes and discount rates. The fair value of the 2021 Notes upon settlement in July 2021 was determined based on the fair value of the Series C-1 redeemable convertible preferred stock issued. This method was selected as we concluded that the contemporaneous financing transaction was an arm's length transaction.

Stock-Based Compensation

We account for stock-based compensation under the provisions of ASC 718-10, Compensation—Stock Compensation, which requires all share-based payments to employees, non-employees and directors, including grants of stock options and restricted stock, to be recognized in the

consolidated statements of operations based on their fair values on the date of grant over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. Generally, we issue stock option awards with only service-based vesting conditions and record the expense for these awards using the straight-line method. We classify stock-based compensation expense in the same manner in which the awards recipient's payroll or service provider's costs are classified.

The fair value of each option grant is estimated on the date of grant using the Black-Scholes option pricing model, which requires inputs based on certain subjective assumptions, including the expected stock price volatility, the expected term of the award, the risk-free interest rate, and expected dividends. We estimate the expected stock price volatility based on the historical volatility of publicly traded peer companies. The expected term of our stock options has been determined utilizing the "simplified" method for awards that qualify as "plain vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. There is no expected dividend yield since we have never paid cash dividends on common stock and do not expect to pay any cash dividends in the foreseeable future.

Common Stock Valuations

As there has been no public market for our common stock, the estimated fair value of common stock has determined by our Board of Directors as of the date of each option grant, with input from management, considering third-party valuations of our common stock as well as our Board of Directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent third-party valuation through the date of the grant. Historically, these independent third-party valuations of our equity instruments were performed contemporaneously with identified value inflection points. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately Held Company Equity Securities Issued as Compensation or the Practice Aid*. The Practice Aid identifies various available methods for allocating the enterprise value across classes of series of capital stock in determining the fair value of our common stock at each valuation date.

The assumptions used to determine the estimated fair value of our common stock are based on numerous objective and subjective factors, combined with management judgment, including:

- external market conditions affecting the pharmaceutical and biotechnology industry and trends within the industry;
- our stage of development and business strategy;
- the rights, preferences, and privileges of our redeemable convertible preferred stock relative to those of our common stock;
- the prices at which we sold shares of our redeemable convertible preferred stock;
- our financial condition and operating results, including our levels of available capital resources;
- the progress of our research and development efforts;
- equity market conditions affecting comparable public companies;
- economic outlook including economic growth, inflation and unemployment, interest rate environment, and global economic trends; and
- the lack of marketability of our common stock

In accordance with the Practice Aid, we determined the hybrid method of the option pricing method, or OPM, and the Probability-Weighted Expected Return Method, or PWERM, was the most appropriate method for determining the fair value of our common stock based on our stage of development and other relevant factors. The OPM uses option theory to value the various classes of a company's securities in light of their respective claims to the enterprise value. Total shareholders' equity value is allocated to the various share classes based upon their respective claims on a series of call options with strike prices at various value levels depending upon the rights and preferences of each class. A Black-Scholes closed form option pricing model is employed in this analysis, with an option term assumption that is consistent with the expected time to a liquidity event and a volatility assumption based on the estimated stock price volatility of a peer group of comparable public companies over a similar term.

The PWERM values each class of equity based on an analysis of the range of potential future enterprise values of the company and the manner in which those values would accrue to the owners of the different classes of equity. This method involves estimating the overall value of the subject company under various liquidity event scenarios and allocating the value to the various share classes based on their respective claim on the proceeds as of the date of each event. These different scenarios typically include an initial public offering, an acquisition, or a liquidation of the business, each resulting in a different value. For each scenario, the future value of each share class is calculated and discounted to a present value. The results of each scenario are then probability weighted in order to arrive at an estimate of fair value for each share class as of a current date.

The hybrid method is a hybrid between the PWERM and OPM, estimating the probability-weighted value across multiple scenarios, but using the OPM to estimate the allocation of value within one or more of the scenarios. In our hybrid method, two types of future event scenarios were considered: an initial public offering, or IPO, and a non-IPO scenario accounting for all other potential future exits. Under both scenarios, the enterprise value was determined at each valuation date using a combination of the cost approach; the income approach, specifically a discounted cash flow analysis; and the market approach, specifically a backsolve to the last round of financing. The relative probabilities between the future exit scenarios were determined by our board of directors based on an analysis of performance and market conditions at the time, including then current IPO valuations of similarly situated companies and expectations as to the timing and likely prospects of future event scenarios.

The assumptions underlying these valuations represented management's best estimates, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could have been materially different.

Following the closing of this offering, our board of directors will determine the fair market value of our common stock based on its closing price as reported on the date of grant on the primary stock exchange on which our common stock is traded.

Internal Control over Financial Reporting

In the course of reviewing our consolidated financial statements in preparation for this offering, our management identified deficiencies that we concluded represented material weaknesses in our internal control over financial reporting attributable to our lack of sufficient financial reporting and accounting personnel. SEC guidance regarding management's report on internal control over financial reporting defines a material weakness as a deficiency or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected and corrected on a timely basis.

Deficiencies in our internal controls over financial reporting that were considered to be a material weakness as of December 31, 2019 were related to the lack of a sufficiently precise review over both valuations prepared by our third-party valuation experts as well as the completeness of operating expenses. We remediated the material weakness related to the review of valuation reports by adding a precise review control that was performed by our accounting personnel with the appropriate technical expertise to review valuation reports. In addition, we have hired an accounting executive with the requisite knowledge in the application of U.S. GAAP and SEC reporting who will be collaborating and reviewing valuation reports prepared by our third-party valuation experts. For the deficiency related to the completeness of operating expenses, we continue to take measures to remediate the deficiency by updating our internal controls to include additional staffing as well as augmenting our controls addressing the completeness of operating expenses, as outlined below.

Further deficiencies in our internal controls over financial reporting that have been considered to be a material weakness as of December 31, 2020 relate to deficiencies in the design of controls over the expenditures process. Specifically, our information technology controls related to the expenditures cycle were not designed to post invoices approved in the correct period, and our controls over the review of the completeness of operating expenses as it related to our close cycle were not appropriately designed, as we lacked sufficient personnel in our Finance and IT organizations to review and provide reasonable assurance that transactions were being recorded timely and completely. We are in the process of implementing changes to our internal controls over financial reporting to remediate this material weakness that has been identified and these changes include hiring a sufficient number of accounting and IT personnel to focus on our information technology systems and to adequately manage the monthly close process.

However, there can be no assurance that we will be successful in pursuing these measures or that these measures will significantly improve or remediate the material weaknesses described above. There is also no assurance that we have identified all of our material weaknesses or that we will not in the future have additional material weaknesses. See “Risk Factors—Risks Related to Our Common Stock and this Offering—If we fail to remediate our material weaknesses in internal controls over financial reporting and to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations, meet our reporting obligations or prevent fraud, and investor confidence in our company and the market price of our common stock may be materially and adversely affected.”

Emerging Growth Company and Smaller Reporting Company Status

The Jumpstart Our Business Startups Act of 2012, or the JOBS Act, permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, we will not be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies, and our consolidated financial statements may not be comparable to other public companies that comply with new or revised accounting pronouncements as of public company effective dates. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

We will cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more, (ii) the last day of our fiscal year following the fifth anniversary of the date of the closing of this offering, (iii) the date on

which we have issued more than $1.0 billion in nonconvertible debt during the previous three years or (iv) the date on which we are deemed to be a large, accelerated filer under the rules of the Securities and Exchange Commission.

We are also a “smaller reporting company”, meaning that the market value of our stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our stock held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements appearing at the end of this prospectus.

BUSINESS

Introduction

Our mission is to develop therapies that change the treatment paradigm for amyotrophic lateral sclerosis, or ALS, and a broad range of neurodegenerative diseases by keeping neurons alive. Unlike most other cells in the body that regularly die and are replaced as part of healthy function, mature neurons are normally resistant to cell death and generally cannot regenerate. We are pursuing commercialization of our product candidate, AMX0035, which we believe is the first drug candidate to show both a functional and survival benefit in a large-scale clinical trial of patients with ALS. We submitted a New Drug Submission, or NDS, in Canada in the second quarter of 2021 for AMX0035 for the treatment of ALS and a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, in the fourth quarter of 2021. We also intend to submit a Marketing Authorization Application, or MAA, in Europe in the first quarter of 2022. The results of our Phase 2 clinical trial of AMX0035, known as the CENTAUR trial, were published in September 2020 in the *New England Journal of Medicine* and in October 2020 in the *Journal of Muscle and Nerve* and demonstrated functional and survival benefits for ALS patients. We believe AMX0035 has the potential to be a foundational therapy, meaning that it could be used alone or in conjunction with other therapies to change the treatment paradigm across a broad range of neurodegenerative diseases.

AMX0035 is a dual UPR-Bax apoptosis inhibitor composed of sodium phenylbutyrate, or PB, and TURSO (also known as tauroursodeoxycholic acid, or TUDCA). Through the resolution of the unfolded protein response, or UPR, and by inhibiting translocation of the Bcl-2 Associated X-protein, or Bax, to the outer mitochondrial membrane, we have shown in multiple models that AMX0035 can keep neurons alive under a variety of different conditions and stresses. We are pursuing ALS as our first indication as it is a disease of rapid and profound neurodegeneration, and we are focused on the development and potential commercialization of AMX0035 for ALS globally.

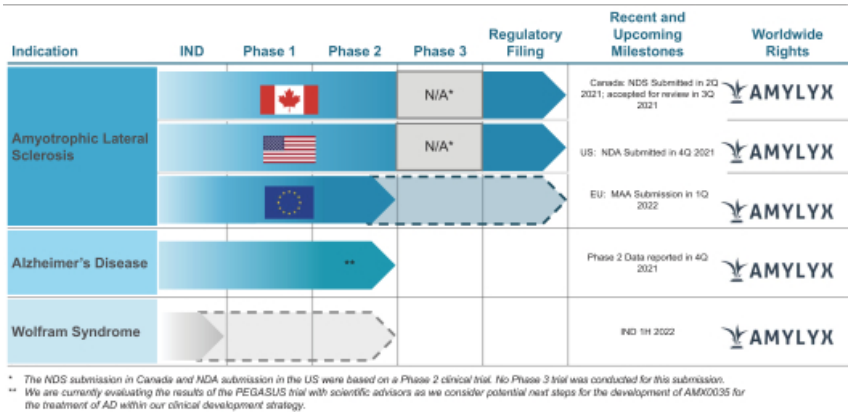
We are actively pursuing regulatory approvals of AMX0035 for the treatment of ALS in Canada, the United States and Europe. We have initiated a Phase 3 clinical trial of AMX0035 for the treatment of ALS at clinical trial sites in the United States and Europe. This trial, known as the PHOENIX trial, is designed to provide further data supporting AMX0035. Based on dialogue with the FDA prior to our NDA submission, including at a pre-NDA meeting recommended by the FDA and subsequent discussions, we believe that data from the PHOENIX trial will not be required for the FDA to make a determination on the approval of AMX0035 for the treatment of ALS, although there can be no assurance that the FDA will not require further data before making a determination. The PHOENIX trial is designed to provide further data supporting the safety and efficacy of AMX0035 for the treatment of ALS to further support our global regulatory efforts.

In addition, we are developing AMX0035 for other neurodegenerative diseases by leveraging our deep knowledge of and relationships in the neurodegenerative space. We believe the approach of a dual UPR-Bax apoptosis inhibitor designed to help keep neurons alive could be clinically meaningful for the treatment of other neurodegenerative disease indications in addition to ALS. Many common and rare neurodegenerative diseases are characterized by substantial neuronal cellular loss, including Alzheimer's disease, or AD, and Wolfram syndrome, as well as Parkinson's Disease, Huntington's Disease, Progressive Supranuclear Palsy, Multi-System Atrophy, and others. We conducted a Phase 2 clinical trial in AD, known as the PEGASUS trial, to obtain safety data along with initial efficacy and biomarker data which could help us prioritize additional indications to pursue with AMX0035. We believe the topline results from the PEGASUS trial, reported in November 2021, provide further biological knowledge about AMX0035 which will help inform future clinical development of AMX0035 for the treatment of AD and in other potential indications. We will continue to evaluate these data and discuss the results of the PEGASUS trial with scientific advisors as we consider potential next steps for the development of AMX0035 for the treatment of AD within our clinical development strategy. Based on preclinical evidence, we are planning to pursue a clinical trial in Wolfram syndrome. We intend to

prioritize our development efforts around neurodegenerative diseases that result in substantial disability, and ultimately death, and where unmet medical needs are greatest.

Since our founding in 2013, our goal has been to improve the quality of, and extend, life for patients suffering from neurodegenerative diseases. One of our key strategies towards achieving this goal has been to form direct relationships with patients, their families, advocacy groups, and healthcare professionals to bring much needed innovation to patients. Throughout the development of AMX0035, we have partnered with members of the disease communities we serve, including the ALS Association, the Northeast ALS Consortium, or NEALS, ALS Finding a Cure, the Healey Center at Massachusetts General Hospital, the Cure Alzheimer's Fund, the Alzheimer's Association and the Alzheimer's Drug Discovery Foundation, to ensure our goals are aligned with patient needs. In addition, many of the key opinion leaders in the ALS community were and are investigators in our recent and ongoing trials. These relationships are a cornerstone of our culture and corporate strategy.

Our current pipeline, including the stage of development of AMX0035 in our target indications, is represented in the table below.



* *The NDS submission in Canada and NDA submission in the US were based on a Phase 2 clinical trial. No Phase 3 trial was conducted for this submission.*
** *We are currently evaluating the results of the PEGASUS trial with scientific advisors as we consider potential next steps for the development of AMX0035 for the treatment of AD within our clinical development strategy.*

AMX0035 is a proprietary oral fixed-dose combination of two small molecules: PB, which is a small molecular chaperone that reduces the UPR, preventing cell death resultant from the UPR, and TURSO, (also known as tauroursodeoxycholic acid, or TUDCA), which is a Bax inhibitor that reduces cell death through apoptosis. While the PB and TURSO molecules individually are not proprietary to us, we own patents and patent applications covering AMX0035, including the fixed-dose combination of AMX0035 itself. We believe that our proprietary combination of these two mechanisms of action will allow us to target abnormal cell death to better prevent neurodegeneration than treatment with either mechanism of action alone. In *in vitro* studies, PB and TURSO were observed in combination to prevent nearly 100% of neuron death. However, PB and TURSO alone only prevented a moderate percentage of neuron death in *in vitro* studies.

The results of our CENTAUR trial were published in September 2020 in the *New England Journal of Medicine* and in October 2020 in the *Journal of Muscle and Nerve*. Trial results showed that patients receiving AMX0035 experienced statistically significant benefit in function, as measured by the Revised ALS Functional Rating Scale, or ALSFRS-R, as well as statistically significant improvement in overall survival, or OS, when analyzing the full randomized population through the open-label extension, or OLE, trial (July 20, 2020 data cutoff). AMX0035 was shown to be generally well-tolerated with the prevalence of adverse events comparable across placebo and treatment groups. We believe AMX0035 is the first drug candidate in ALS to demonstrate a statistically significant benefit both in function as measured by a prespecified mean rate change in ALSFRS-R and in a longer-term analysis of OS, which are both important outcomes for people with ALS.

Our Company and Team

Amylyx was founded with the ambitious goal of improving the quality and length of life for patients suffering from neurodegenerative diseases. From a dorm room at Brown University in 2013, Co-CEOs and Co-Founders Josh Cohen and Justin Klee set out to determine why neurons die, and have been working ever since to develop AMX0035, which we believe is the first drug candidate to show a prespecified and statistically significant effect on function and survival in ALS, and other novel therapies. To help realize our goal, we have assembled a team with deep scientific, clinical, business and leadership experience, bolstered by expertise in biotechnology. Our Chief Financial Officer, James Frates, brings over 20 years of experience as the Chief Financial Officer of Alkermes. Our Chief Commercial Officer, Margaret Olinger, brings three decades of expertise in commercial launches and operations, most recently at Alexion. Our Global Head of Supply, Tom Holmes, brings more than 25 years of leadership experience at Biogen in supply chain, biopharmaceutical manufacturing and program management. Our Global Head of Clinical Research & Development and Chief Medical Officer, Patrick D. Yeramian, brings over 30 years of medical and pharmaceutical industry experience. Our Head of Regulatory Affairs, Tammy Sarnelli, brings more than 30 years of experience from Biogen and other companies in early and late-stage neurology and rare disease development. Our Global Head of Human Resources, Debra Canner, brings over 20 years of experience as a human resources leader, having served at Akamai Technologies and Genzyme. This team brings a diverse set of skills uniquely suited to drive successful commercialization of AMX0035 in ALS while continuing to advance AMX0035 in other indications.

Our Strategy

Our mission is to change the treatment paradigm for neurodegenerative diseases. Key elements of our strategy to achieve this mission include:

- ***Obtaining regulatory approval of AMX0035 for ALS in Canada, the United States and Europe.*** Based on the results from our CENTAUR trial, we have been exploring pathways towards regulatory approval in several territories, including Canada, the United States and Europe. We believe that the CENTAUR trial may be able to support regulatory approval in Canada and the United States and marketing authorization in Europe. We submitted an NDS in Canada in the second quarter of 2021 and an NDA in the United States in the fourth quarter of 2021, and plan to submit an MAA in Europe in the first quarter of 2022. We initiated our global Phase 3 PHOENIX trial of AMX0035 for the treatment of ALS in the fourth quarter of 2021 to further support our global regulatory efforts.
- ***Effectively and efficiently commercializing AMX0035 for ALS in key territories, if approved.*** We submitted an NDS in Canada in the second quarter of 2021 and an NDA in the United States in the fourth quarter of 2021, and also intend to submit an MAA in Europe in the first quarter of 2022, and subject to receiving marketing approval, we would expect to launch AMX0035 for ALS in Canada in , in the United States in and in Europe in . We are currently building our sales team, internal capabilities and outside vendor network to support commercialization. We believe these capabilities, coupled with our understanding of the ALS patient and medical community, will enable us to launch AMX0035 for ALS successfully, if approved. We anticipate that our commercial infrastructure will be scalable for subsequent launches in the United States and other key markets if we receive marketing approval in these territories as well. Our commercial operations are led by Margaret Olinger, who was an early commercial employee at Alexion and helped lead the launches of Soliris for the treatment of generalized myasthenia gravis and Strensiq for the treatment of perinatal/infantile- and juvenile-onset hypophosphatasia.
- ***Maximizing the therapeutic potential of AMX0035 by expanding into additional neurodegenerative diseases.*** We believe the data from the CENTAUR trial showing a

functional and survival benefit for ALS patients treated with AMX0035 validates its mechanism of targeting endoplasmic reticulum, or ER, stress and mitochondrial dysfunction. Based on our extensive understanding of disease pathways, we believe AMX0035 may provide benefit across multiple diseases characterized by neurodegeneration. As we select the next indications for AMX0035 we will prioritize those indications which we believe, if successful, will most rapidly lead to marketed products and to patient benefit. We conducted our Phase 2 PEGASUS clinical trial in AD to obtain safety data along with initial efficacy and biomarker data which will help us evaluate the development of AMX0035 for the treatment of AD within our clinical development strategy. We expect to submit an IND for AMX0035 in Wolfram syndrome in the first half of 2022. We expect to submit an IND for two additional indication in 2022.

- ***Continuing to cultivate a network of patient advocacy groups, key opinion leaders, research institutions, and healthcare professionals to inform our patient-centric approach.*** We have cultivated a network of key constituents, which we believe will continue to help us to develop therapies in an efficient and impactful manner. Integrating the experiences and insights from these parties, which include patients, their families, and organizations such as the ALS Association, NEALS, ALS Finding a Cure, the Healey Center at Massachusetts General Hospital, the Cure Alzheimer's Fund, the Alzheimer's Association and the Alzheimer's Drug Discovery Foundation, continues to inform our approach to developing therapies that can potentially transform the lives of patients and their families. We intend to will continue to engage with each of these constituents through conferences, clinical trials and informal communications as we further develop and pursue commercialization of AMX0035.
- ***Deploying a strategic approach to design, acquire and develop new therapies.*** We follow a scientifically rigorous approach to evaluating new opportunities to broaden our portfolio. We plan to target assets that allow us to leverage our experience with neurodegenerative pathways and AMX0035's mechanism of action, focusing primarily on preventing neuron death. When evaluating assets, we consider not only our ability to apply our experience with AMX0035 but also a variety of factors, including unmet medical need, biological rationale, feasibility of clinical development, potential for regulatory approval, costs of development, competitive landscape and commercial potential.

Neurodegenerative Disease

The prevention of neurodegeneration represents one of today's most significant unmet medical needs. The development of therapies that preserve neuron health has historically presented unique challenges, including an imperfect understanding of underlying biology and a lack of translation of activity observed in preclinical studies to results in clinical trials. Currently approved therapies for many neurodegenerative diseases are generally only symptom modifying and have demonstrated limited efficacy. There remains an urgent need for novel approaches to address most neurodegenerative diseases, especially for progressive and severe conditions such as ALS.

The Role of the Endoplasmic Reticulum and Mitochondria in Neurodegenerative Disease

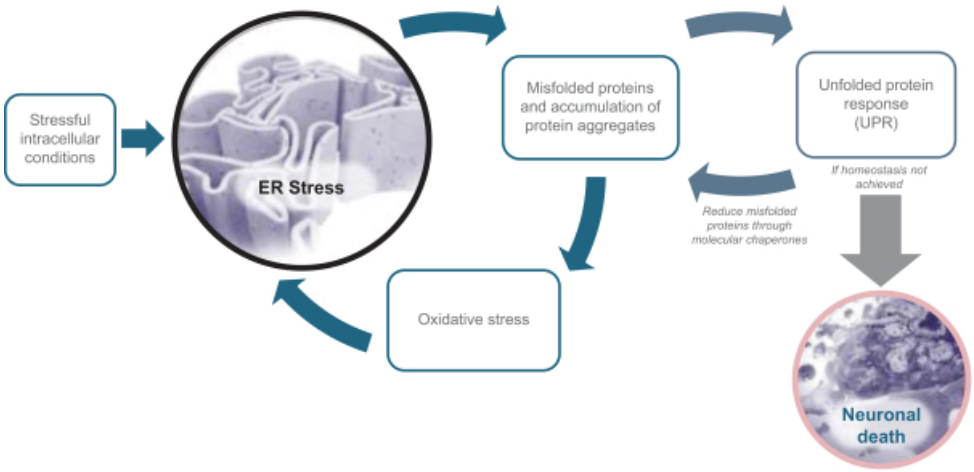
Unlike most other cells in the body that regularly die and are replaced as part of healthy function, mature neurons are normally resistant to cell death and generally cannot regenerate. Neuron death is only triggered when multiple stress factors are activated beyond the neuron's recovery capacity, a circumstance commonly seen in neurodegenerative disorders. Most neurodegenerative disorders have complex pathophysiology, with multiple pathways contributing and converging to eventually cause neuron death. A large fraction of these pathological changes in neurons can be linked to dysfunction in the ER and mitochondria that affect metabolism and secretion of lipids and proteins, calcium homeostasis, and energy production. Dysfunction in these two essential cellular structures is implicated across many neurodegenerative disorders, highlighting the central role they play in

maintaining neuron health and survival and providing the rationale for our focus, which is to rescue ER and mitochondrial function, and to protect and preserve neurons.

ER Stress

The ER is responsible for protein and lipid synthesis, folding and quality control of proteins, and storing calcium for cellular energy production by the mitochondria. The ER is also a primary sensor of stressful intracellular conditions, activating a wide number of molecular pathways that belong to a specific process, referred to as the ER stress response, that controls protein homeostasis. ER stress, or dysfunction associated with protein misfolding and aggregation, has been implicated in the pathogenesis of neurodegenerative disease. In neurodegenerative disorders, misfolded proteins and accumulations of protein aggregates can cause oxidative stress and a feedback loop resulting in ER stress. When the ER stress response is activated due to misfolded and aggregated proteins, the UPR, is engaged as a regulatory mechanism to reduce the load of misfolded proteins and restore a healthy cellular state. Molecular chaperones are the critical regulators of protein homeostasis under ER stress. Pathological conditions such as neurodegenerative diseases that disturb protein folding and maturation can trigger ER stress and engage the UPR. When the natural protein homeostasis in the cell cannot be achieved, the UPR triggers cellular death, or apoptosis, as illustrated in the graphic below.

The Role of the ER in Neurodegenerative Disease



Mitochondrial Dysfunction

The mitochondria are a central regulatory point for the control of cell death. When mitochondria detect sufficient cell damage, they signal for the cell to initiate a cell death cascade. Among other steps, this cascade includes the recruitment of a series of apoptotic proteins including Bcl-2-associated X protein, or BAX, the release of cytochrome c from a pore in the mitochondrial membrane called the mitochondrial permeability transition pore, and finally the activation of caspase 3, an executioner protein for apoptosis.

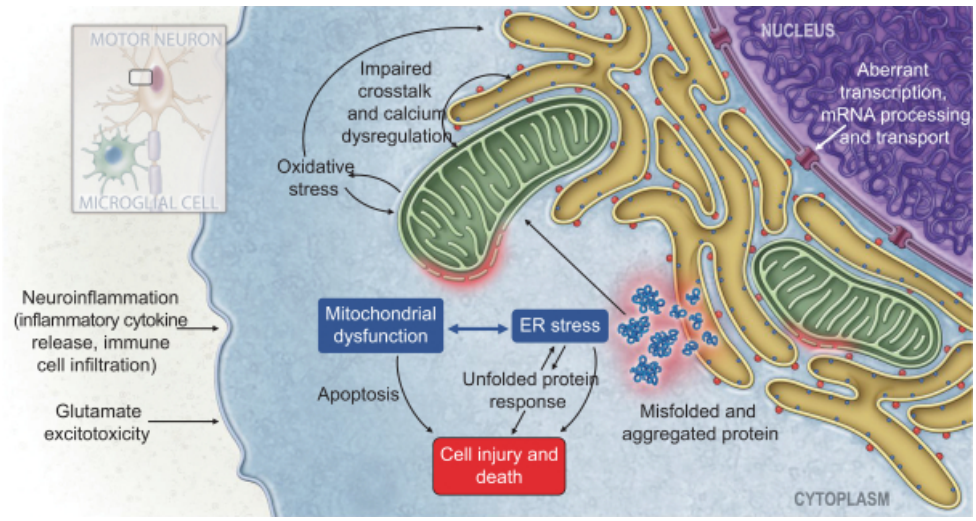
In neurodegenerative diseases, triggers such as altered calcium homeostasis, glutamate excitation of the cell, damage to the mitochondria or mitochondrial DNA and detection of aberrant double-stranded DNA and accumulation of unfolded proteins at the mitochondria all lead to mitochondrially mediated cell death. Inhibition of proteins such as BAX could result in a greater threshold for cell death and longer survival of key neurons implicated in the progression of neurodegenerative disease.

Linkage Between Mitochondria and ER

The mitochondria and the ER are often physically linked by a membrane called the mitochondrial associated ER membrane, or MAM. Through this linkage, calcium and molecules are shuttled between the two organelles. It is our belief that this connection, or crosstalk, allows the cell to integrate responses between the two organelles and that activation of mitochondrial damage pathways will activate the UPR and *vice versa*.

Both the mitochondria and the UPR in the ER can trigger cell death. As such, we believe both pathways are crucial to the pathogenesis of neurodegenerative diseases and both need to be addressed simultaneously to effect a substantial change in survival of neurons undergoing neurodegenerative processes.

The Role of the ER and Mitochondria in Neuron Death



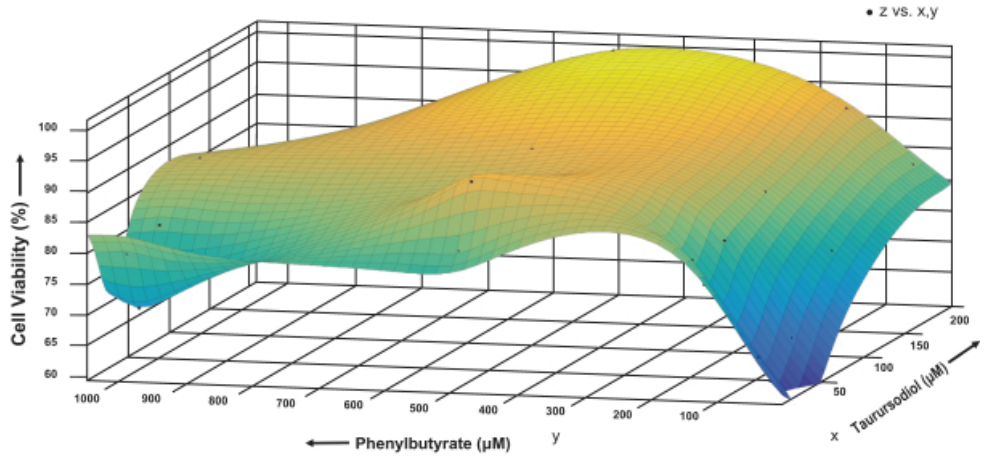
The figure above depicts events of ER stress and mitochondrial dysfunction associated with eventual cell injury and death.

Background and Rationale for AMX0035

We have designed AMX0035 to reduce neuron death through simultaneous mitigation of ER stress and mitochondrial dysfunction. AMX0035 is a coformulation of two small molecules, PB and TURSO. PB has been shown to reduce ER stress through upregulation of a protein known as DJ-1 that is a master

chaperone regulator, recruitment of other chaperone proteins, and as a small molecular chaperone. TURSO is a bile acid that has been shown to recover mitochondrial bioenergetic deficits through incorporation into the mitochondrial membrane, reducing BAX translocation to the mitochondrial membrane, reducing mitochondrial permeability, and increasing the apoptotic threshold of the cell. Through our research, we identified the specific ratios at which the combination of PB and TURSO target these critical, connected pathways and show synergistic activity in improving neuronal cell viability *in vitro*. We then developed AMX0035 as an optimized oral formulation to be tested *in vivo* and clinically.

Our preclinical studies have shown that PB and TURSO, in combination, can inhibit a number of pathological pathways associated with neurodegenerative diseases in cell culture and animal models. For example, in an *in vitro* model of neurodegeneration, we tested the potential abilities of PB and TURSO individually and in combination to prevent oxidative-induced neuronal death, or cell viability, which was measured using a PrestoBlue reagent. In this experiment, hydrogen peroxide was applied to rat primary cortical neurons in a concentration sufficient to kill approximately 40% of the neurons. Particular doses of PB and TURSO individually protected against some of the neuron death, and cell viability reached approximately 80%. However, when these rat primary cortical neurons were dosed with particular ratios of PB and TURSO in combination, nearly 100% of oxidative-induced neuron death was prevented. The results of this *in vitro* model are shown in the graphic below.



Additionally, we have observed benefit from the administration of particular ratios of PB and TURSO across *in vitro* models of ER stress, mitochondrial dysfunction, oxidative stress, and disease specific models of ALS, AD, Parkinson's disease, multiple sclerosis, or MS, Friedreich's Ataxia, primary mitochondrial myopathies and a variety of other conditions. We have also conducted *in vivo* models of PB and TURSO, in combination, including models of ALS, AD and MS. Additionally, academic groups have conducted studies with monotherapy treatment with TURSO and/or PB in models of ALS, AD, MS, Parkinson's Disease, Huntington's Disease, Progressive Supranuclear Palsy, Multi-System Atrophy, X-linked adrenoleukodystrophy, and a variety of other models. We believe this body of evidence collectively supports the use of this combination to treat neurodegenerative indications and led us to pursue the development of our proprietary drug candidate, AMX0035.

AMX0035 for the Treatment of ALS

Overview of ALS

We are initially developing AMX0035 for the treatment of ALS, an adult-onset, progressive, and fatal neurodegenerative disorder of the neuromuscular system resulting in muscle weakness and paralysis leading to death. ALS involves the progressive degeneration of motor neurons in the spinal cord and brain that are responsible for controlling voluntary muscle movement. This progressive loss of motor neurons leads to muscle weakness, loss of muscle mass, and inability to control movement. ALS remains universally fatal with a median survival of less than three years from symptom onset and less than two years from diagnosis. Despite being classified as a rare disease by the FDA and the EMA, ALS is considered one of the more common adult-onset neuromuscular diseases worldwide. Public sources estimate approximately 25,000 prevalence ALS patients in the United States. We believe the prevalence is closer to 30,000 in the United States based on research that we have conducted in collaboration with expert consulting groups. Approximately 40,000 ALS patients are estimated to be located in Europe and about 3,000 ALS patients are located in Canada. Over 90% of patients have no family history of ALS, known as “sporadic” ALS. While other development approaches seek to address genetic instances of ALS, AMX0035 is designed to target all instances of ALS, regardless of whether it is sporadic or genetic. Due to the two-year median survival of patients diagnosed with ALS, a high proportion of the patient population has been recently diagnosed and a therapy that is able to improve the survival of patients with ALS has the potential to increase the number of patients who are able to continue living with their disease.

Medical costs for patients newly diagnosed with ALS in the United States are substantial and increase rapidly with each disability milestone. Care of patients with ALS is intensive and requires a team of medical professionals, special equipment, and assistance with daily activities. Caregivers are often forced to miss work or give up employment opportunities to provide care, leading to increased financial strain. The disease also impacts the patient’s family, who generally provide the bulk of caregiving, which often entails the provision of 24-hour care. The constant adaptation of caregivers to the demands of the ALS disease progression requires significant physical effort and mental exhaustion particularly during the advanced stages of the disease.

Significant Unmet Need in ALS

ALS is a heterogeneous disease that arises from multiple mechanistic underpinnings, leading patients to experience variable onset and delayed diagnosis, persistent progression and loss of muscle function, and shortened survival.

There is a significant unmet need for ALS therapies that target multiple pathogenic pathways, are disease-modifying, and can provide both functional and survival benefit to patients. Only two FDA-approved therapeutic agents for ALS, riluzole, an anti-glutamatergic agent, and edaravone, a free-radical scavenger, have been shown to modulate the course of ALS. In pivotal clinical trials, riluzole demonstrated longer time to tracheostomy or death compared to placebo and edaravone demonstrated longer retention of function compared to placebo. However, a need remains for ALS therapies that demonstrate both retention of function and longer survival, allowing patients to maintain greater independence for longer.

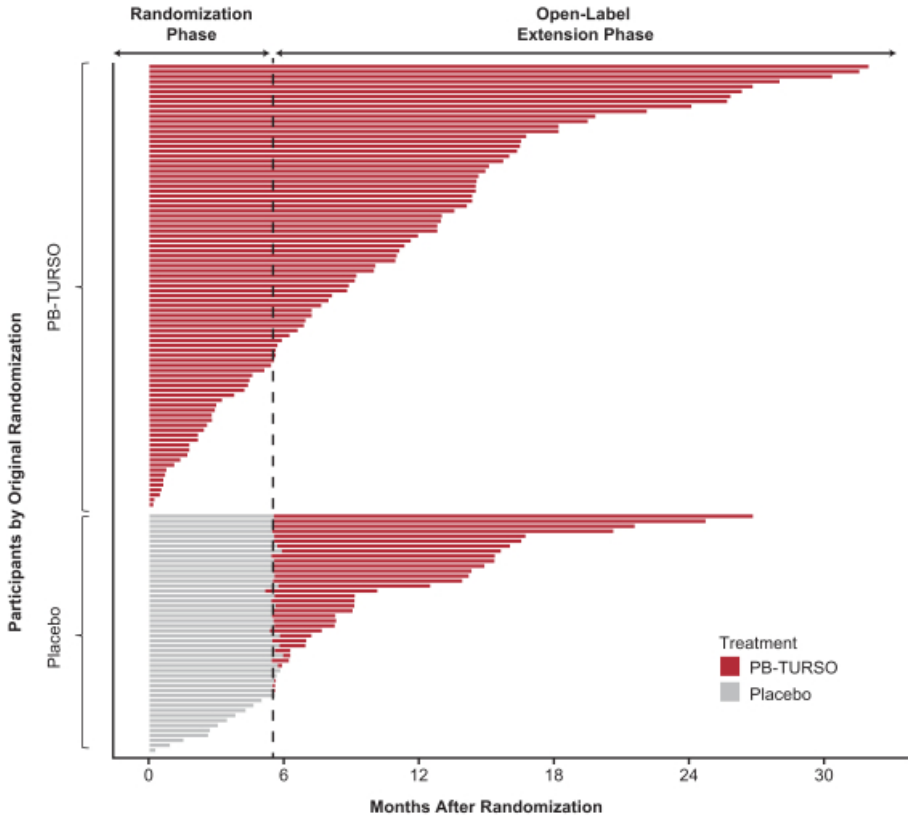
Due to the multi-pathway pathophysiology of ALS, experts agree that successful treatment will likely require concurrent targeting of multiple key neuronal death pathways. There is a strong rationale for treatments that target identified convergence points of these critical pathways, including in the ER and mitochondria, and we believe that a therapy that targets multiple pathways at once, like AMX0035, aligns with the emerging ALS treatment paradigm.

Clinical Development of AMX0035 for ALS

We designed our Phase 2 CENTAUR trial with input from leading ALS experts from NEALS to detect a significant difference between AMX0035 and placebo. The study also provided the option for participants to continue with available approved therapies, riluzole and edaravone, for the duration of the trial. The FDA granted Orphan Drug Designation for AMX0035 for the treatment of patients with ALS in September 2017. The EMA granted orphan designation to AMX0035 for the treatment of patients with ALS in April 2020. In December 2019, we announced positive topline results from our CENTAUR trial. The trial met its primary endpoint, and we published detailed trial data in the *New England Journal of Medicine* in September 2020 and in the *Journal of Muscle and Nerve* in October 2020. We submitted an NDS in Canada in the second quarter of 2021 and submitted an NDA in the United States in the fourth quarter of 2021. We also intend to submit an MAA in Europe in the first quarter of 2022.

CENTAUR, Our Phase 2 Trial of AMX0035 in ALS

In September 2020, we published detailed results from the Phase 2, randomized, double-blind, placebo-controlled CENTAUR trial. The CENTAUR trial was conducted at 25 centers of the NEALS, and evaluated adult patients with ALS. Key inclusion criteria were definite ALS defined by the revised El Escorial criteria, which entails having various clinical signs and symptoms, defined as upper and lower motor neuron signs, in at least three defined body regions, less than 18 months from symptom onset and slow vital capacity, or SVC, greater than 60%. These criteria were chosen to select a homogenous, rapidly progressing patient population to potentially increase the likelihood of observing a treatment effect. Participants were allowed to continue on their selected standard of care, including treatment with riluzole and/or edaravone. Eligible participants (n=137) were randomized two-to-one to treatment with AMX0035, one sachet (each containing one gram of TURSO and three grams of PB) given once daily for the first three weeks, and if tolerated, the dose was then increased to twice-daily for the remainder of a 24 week treatment period, or matching placebo. Two participants did not have follow-up efficacy assessments and were not included in the efficacy population (modified intention to treat, or mITT, n=135). These two participants were included in the safety population (intention to treat, or ITT, n=137). Upon completion of the 24-week, parallel group phase of the trial, participants were eligible to enroll in the OLE trial in which all participants were followed up to 35 months while participants and physicians remained blinded to the original treatment group. Of participants completing the CENTAUR trial randomization phase, 92% elected to enroll in the OLE. The OLE was completed in March 2021. Actual duration of patient treatments across the randomization phase and the OLE, both with the PB-TURSO combination and via placebo, are shown in the graphic below:

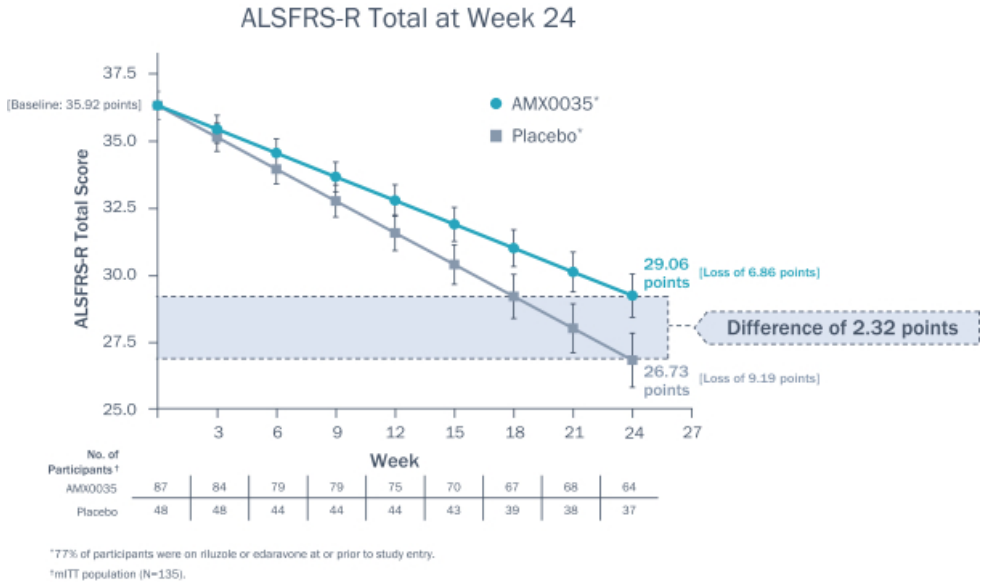


The primary efficacy outcome measure for the CENTAUR trial was the rate of decline in the ALSFRS-R total score. The ALSFRS-R scale is the most widely used ALS rating scale in ALS clinical practice and in ALS clinical trials. It measures patient's functional ability and is broken down into four domains: bulbar (which includes speech, salivation and swallowing), fine motor (which includes handwriting, cutting food/handling utensils, dressing and hygiene), gross motor (which includes turning in bed, walking, and climbing stairs) and breathing (which includes dyspnea, orthopnea and respiratory insufficiency). A decrease of one point on the ALSFRS-R scale can reflect severe limitations in a patient's independence, and a two-point increase on the ALSFRS-R scale would be associated with:

- eating successfully with some difficulty instead of needing a feeding tube;
- being short of breath only while walking instead of having difficulty breathing while sitting or lying down; and
- being able to dress independently instead of needing assistance.

The CENTAUR trial met its primary endpoint with a statistically significant slowing of functional decline among participants randomized to AMX0035 (n=87) compared to placebo (n=48) (p-value of 0.03) over 24 weeks. These results showed that patients receiving AMX0035 scored an average of 2.32 points higher on the ALSFRS-R as compared to patients receiving placebo after 24 weeks, a difference of 25%, as shown in the graph below. In a survey of ALS clinicians and researchers conducted and sponsored by NEALS, with the objective of determining what percentage reduction in ALSFRS-R would

be considered clinically meaningful, a difference of greater than or equal to 20% in ALSFRS-R total score was considered clinically meaningful by a majority of clinicians and researchers surveyed.



Secondary efficacy outcomes measuring disease free progression were the decline in muscle strength as measured by Accurate Test of Limb Isometric Strength, or ATLIS, testing and lung function measured by SVC, both expressed as percent of predicted values and key study events including death, permanent ventilation and hospitalization. Neurofilament was also measured as a biologic measure. The analysis also indicated statistically significant preservation of upper limb strength with AMX0035 treatment measured on ATLIS (p=0.042), while the lower limb measure did not reach statistical significance (p=0.34). An average of these two, referred to as the total ATLIS score, trended in favor of AMX0035 (p=0.11). There was also a trend in favor of AMX0035 therapy preserving lung function as measured by SVC, with a numerical difference of 5.11% although this was not statistically significant (p=0.076). These efficacy data are summarized in the table below. In addition, a time-to-event analysis was conducted on key study events including death, permanent ventilation and hospitalization events over the 24-week randomized phase of the trial. Because enrollment of patients in the CENTAUR trial was limited to patients who, in the investigator's opinion, would be able to complete 6-month follow up, few events of this nature were expected during the initial, 24-week randomized phase of the trial. As a result, we observed a positive, but not statistically significant, difference between the trial's treatment and control groups during the 24-week randomized phase of the study. There was no statistically significant difference between the rate of decline in plasma levels of the neurofilament observed in the trial's treatment and control groups during the 24-week randomized phase of the study.

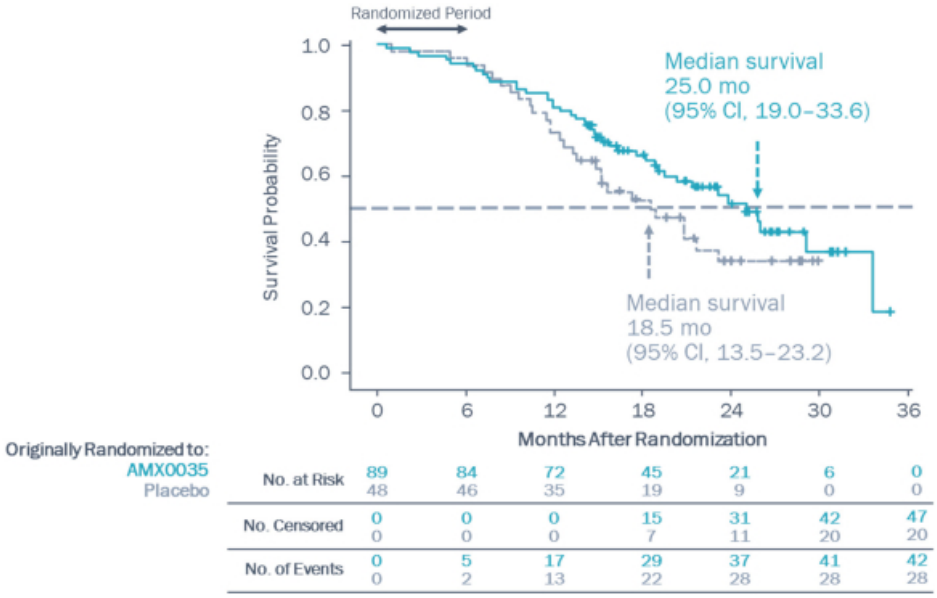
CENTAUR Trial Data

	Estimate (Standard Error)		Difference			
Endpoint Time Point	**Placebo+SOC (n=48)**	**AMX0035+SOC (n=87)**	**Week 24 Difference (Standard Error)**	**95% Confidence Interval**	**p-value**	**Weeks Function Retained (%)**
ALSFRS-R Total Week 24	26.73 (0.975)	29.06 (0.781)	2.32 (1.094)	0.18, 4.47	0.0340	6.1 (33.9%)
SVC % Predicted Week 24	61.06 (2.812)	66.17 (2.327)	5.11 (2.872)	-0.54, 10.76	0.0763	5.5 (29.8%)
Upper ATLIS Week 24	32.36 (2.590)	36.63 (2.316)	4.27 (2.089)	0.16, 8.38	0.0420	4.9 (25.4%)
Lower ATLIS Week 24	39.09 (2.664)	41.17 (2.371)	2.09 (2.195)	-2.23 6.41	0.3424	2.7 (12.7%)

Phosphorylated neurofilament heavy chain was measured in plasma in the CENTAUR trial. There were no statistically significant differences between groups in this outcome. A limitation of this outcome is that it was measured in plasma rather than cerebrospinal fluid and the ultimate relevance of this outcome in ALS is still under investigation by the field.

It is important to note that most (77%) participants were receiving riluzole or edaravone at or before study entry, with a greater proportion receiving edaravone in the placebo group (50%) compared with the AMX0035 group (25%). Pre-specified analyses were conducted to determine if the use of concomitant medications impacted results. These analyses found that AMX0035's effect on the primary outcome was consistent regardless of baseline use of concomitant medications (riluzole and/or edaravone).

OS was analyzed for all subjects randomized in the CENTAUR trial (ITT analysis) and compared patients originally randomized to AMX0035 (n=89) with those randomized to placebo (n=48). In this analysis, the vital status of each participant was measured by a participant locating service which used sources such as the U.S. social security death index up to July 20, 2020 even if he or she did not continue into the OLE, stopped study drug, dropped out of the study or was lost to follow-up. Over the duration of follow up, the risk of death was 44% lower among those originally randomized to AMX0035 compared with those originally randomized to placebo (hazard ratio, or HR, of 0.56; a 95% confidence interval, or CI, ranging from 0.34 to 0.92; and a p-value of 0.023). Median survival duration was 25.0 months (95% CI of 19.0 to 33.6 months) in the group previously randomized to AMX0035 and 18.5 months (95% CI of 13.5 to 23.2 months) in the group previously randomized to placebo as seen in the graph below. Participants originally randomized to AMX0035 received a median 6.9 months more of AMX0035 treatments than those originally randomized to placebo.



AMX0035 was generally well-tolerated with an adverse event rate substantially similar to placebo. Adverse events, or AEs, were reported in 97% (86 out of 89) of participants receiving AMX0035 and 96% (46 out of 48) of participants receiving placebo, with the nature of the AEs being substantially similar in both groups. The most commonly occurring (greater than or equal to 5%) AEs in either treatment group are shown in the table below. Because of the progressive neurodegenerative nature of

ALS, many of these AEs (*e.g.*, muscle weakness, falls, dyspnea, fatigue) were likely attributable to the underlying ALS disease. Events occurring in greater than or equal to 5% of patients in either treatment group and more frequently (greater than or equal to 2% of patients) in patients who received AMX0035 compared with those who received placebo were predominantly gastrointestinal events, which were non-serious and mostly mild in intensity and declined considerably in occurrence after three weeks on treatment.

The most commonly occurring AEs were diarrhea, constipation, nausea, muscular weakness, fall, headache, dizziness and viral upper respiratory tract infection. Health Canada also noted the occurrence of hypersalivation. Consistent with the known safety profile of TURSO, diarrhea and nausea occurred more frequently in patients who received AMX0035 compared with those who received placebo. In contrast, muscular weakness, fall, constipation and headache occurred more frequently in patients who received placebo. The observed AEs from the CENTAUR trial are summarized in the chart below.

Adverse Events (AEs)(1) Occurring in ≥5% of Patients in either Treatment Group (Safety Population, n=137)

MedDRA System Organ Class Preferred Term	Placebo + SOC (n=48)	AMX0035 + SOC (n=89)	Overall (n=137)
Gastrointestinal disorders	29 (60.4%)	60 (67.4%)	89 (65.0%)
Musculoskeletal and connective tissue disorders	21 (43.8%)	38 (42.7%)	59 (43.1%)
Injury, poisoning and procedural complications	23 (47.9%)	35 (39.3%)	58 (42.3%)
Nervous system disorders	19 (39.6%)	33 (37.1%)	52 (38.0%)
Infections and infestations	21 (43.8%)	28 (31.5%)	49 (35.8%)
Respiratory, thoracic and mediastinal disorders	10 (20.8%)	29 (32.6%)	39 (28.5%)
Investigations	10 (20.8%)	26 (29.2%)	36 (26.3%)
General disorders and administration site conditions	13 (27.1%)	20 (22.5%)	33 (24.1%)
Skin and subcutaneous tissue disorders	8 (16.7%)	16 (18.0%)	24 (17.5%)
Psychiatric disorders	9 (18.8%)	14 (15.7%)	23 (16.8%)
Renal and urinary disorders	8 (16.7%)	10 (11.2%)	18 (13.1%)
Metabolism and nutrition disorders	4 (8.3%)	10 (11.2%)	14 (10.2%)
Cardiac disorders	0 (0.0%)	7 (7.9%)(2)	7 (5.1%)
Eye disorders	1 (2.1%)	5 (5.6%)	6 (4.4%)

(1) Includes serious adverse events.
(2) There was no direct evidence that any of the cardiac disorder AEs were related to AMX0035. Two events were considered possibly related to AMX0035, while all other events were considered unlikely to be or not related to AMX0035 by the trial's principal investigator. All of the cardiac AEs were asymptomatic and rated mild, as defined by the Common Terminology Criteria for Adverse Events, except for one instance of cardiac arrest, for which there was a convincing alternate explanation.

Serious adverse events, or SAEs, occurred less frequently in the AMX0035 treatment group (12.4% of patients) compared with the placebo treatment group (18.8% of patients). This difference was largely driven by a higher incidence of respiratory events, including respiratory failure in the placebo treatment group (8.3% of patients), compared with the AMX0035 treatment group (3.4% of patients). ALS disease progression often leads to respiratory failure, and it is the most common cause of death in patients with ALS.

Overall, a total of seven patients (two (4% of patients) who received placebo and five (6% of total patients) who received AMX0035) died during the conduct of the 24-week, double-blind study. None of the deaths was considered by the investigator to be related to AMX0035. Consistent with the most

common cause of death in patients with ALS, the majority (four of seven patients) of deaths during the study were from respiratory failure (two patients in each group). Other causes of death (in the AMX0035 group) included post-extubational supraglottic and infraglottic aspiration (attributed to aspiration pneumonia), diverticulitis, and subdural hematoma secondary to a fall. Death equivalent was defined as either tracheostomy or permanent assisted ventilation, or PAV. PAV was defined as more than 22 hours daily of non-invasive mechanical ventilation for more than one week (seven days). One patient in the placebo group (2% of patients) and none in the AMX0035 group experienced a death equivalent event (i.e., tracheostomy) during the 24-week study.

We believe AMX0035 is the first drug candidate in ALS to demonstrate a statistically significant benefit both in function as measured by a prespecified mean rate change in ALSFRS-R and in a longer-term analysis of OS, both important outcomes for people with ALS. In summary, patients in our CENTAUR trial showed a statistically significant improvement in function and a statistically significant improvement in overall survival and AMX0035 was shown to be generally well-tolerated.

Clinical Development Plan of AMX0035 in ALS

We submitted an NDS for AMX0035 for the treatment of ALS to Health Canada in the second quarter of 2021, which was accepted for review in the third quarter of 2021, and an NDA to the FDA in the fourth quarter of 2021. We anticipate that the next steps in the clinical development plan of AMX0035 in ALS will be as follows:

- We intend to submit an MAA for approval of AMX0035 for the treatment of ALS to the EMA Committee for Medicinal Products for Human Use in the first quarter of 2022.
- We recently initiated our global 48-week PHOENIX, randomized, double-blind, placebo-controlled trial in the fourth quarter of 2021. We expect to recruit approximately 600 patients from 55 European and U.S. sites. The primary endpoint our PHOENIX trial will be a composite measure of survival and ALSFRS-R total score progression over 48 weeks. Based on dialogue with the FDA prior to our NDA submission, including at a pre-NDA meeting recommended by the FDA and subsequent discussions, we believe that data from the PHOENIX trial will not be required for the FDA to make a determination on the approval of AMX0035 for the treatment of ALS, although there can be no assurance that the FDA will not require further data before making a determination. The PHOENIX trial is designed to provide further data supporting the safety and efficacy of AMX0035 for the treatment of ALS to further support our global regulatory efforts. Because any marketing approval we may ultimately obtain in Europe and Canada may be limited, subject to restrictions or conditional on post-approval commitments, we may need to provide post-marketing support in those jurisdictions.

Any regulatory approvals we may receive may be limited or subject to restrictions or postapproval commitments.

AMX0035 for the Treatment of Other Potential Indications

Based on our extensive understanding of disease pathways, we believe AMX0035 may provide benefit across multiple diseases, including AD, Wolfram syndrome, Parkinson’s Disease, Huntington’s Disease, Progressive Supranuclear Palsy, Multi-System Atrophy, primary lateral sclerosis, ischemic stroke, MS, Friedreich’s ataxia, Leigh’s syndrome and Leber’s hereditary optic neuropathy.

We are prioritizing these conditions on an indication-by-indication basis, based on the strength of the data supporting AMX0035’s potential, including the data from our recently completed PEGASUS trial; the urgency of the unmet need; the practicality of conducting clinical trials in these conditions; the efficiency of clinical development activities; and the commercial potential. For some of these indications, given the data already produced by the company on AMX0035, we believe it may be possible to move directly into Phase 3 evaluations of safety and efficacy which could allow for a rapid

development pathway. We will prioritize those indications which we believe have the greatest chance of providing patients with benefit and the most rapid pathway to market.

We plan to submit an IND for AMX0035 in Wolfram syndrome in the first half of 2022. Subject to acceptance of this IND, we plan to initiate a proof of concept Phase 2 clinical trial in Wolfram syndrome. Additionally, we plan to submit potential additional INDs for AMX0035 in other indications in the first half of 2022.

Clinical Development of AMX0035 for AD

We designed our multicenter, randomized, double-blind, placebo-controlled Phase 2 PEGASUS trial with AD experts to evaluate the safety, tolerability and activity of AMX0035 in patients with late mild cognitive impairment or early-to-moderate dementia. The PEGASUS trial was designed to have broad entry criteria to include participants at different stages of AD to allow us to assess the biological effect of AMX0035 across the spectrum of disease and determine if there are any patients who might see a greater benefit from therapy. Eligible participants (n=95), adults ages 55 to 89 years old, were randomized three-to-two to treatment with AMX0035, one sachet (each containing one gram of TURSO and three grams of PB) given twice-daily over 24 weeks, or matching placebo.

The primary endpoints of the PEGASUS trial were safety and tolerability and a newly developed global statistical test, or GST, of disease progression, which measures a novel composite outcome of function, cognition and brain atrophy. Exploratory endpoints included biomarkers of AD, neuroinflammation, neurodegeneration and metabolism and oxidative stress.

The primary investigator for the PEGASUS trial, Dr. Steven Arnold, presented topline results from the PEGASUS trial at the Clinical Trials on Alzheimer's Disease conference, which was held during the fourth quarter of 2021. Based on these topline results, AMX0035 met the PEGASUS trial's primary endpoint of safety and tolerability. The 6-month trial was not powered to evaluate differences between groups in efficacy outcomes and no differences were seen in the primary efficacy endpoint, a newly developed composite outcome of cognitive, functional, and imaging measures, or additional efficacy endpoints of cognition, function, and imaging. AMX0035 was observed in this trial to have had a significant impact on multiple biomarkers of interest in AD. In cerebrospinal fluid, or CSF, AMX0035 showed significant reduction of tau protein 181 (p-value of less than 0.001), phosphorylated tau protein (p-value of less than 0.001), modulation of the amyloid beta 42/40 ratio (p-value of less than 0.05) and increase of 8-hydroxy-2' -deoxyguanosine, (p-value of less than 0.01). These topline results from the PEGASUS trial are still subject to further audit and verification procedures. Multiple additional CSF analyses are ongoing to analyze further biomarker changes. We believe this data provides further biological knowledge about AMX0035 which will help inform future clinical development of Amylyx for the treatment of AD, as well as in additional potential indications.

We believe the biomarker and imaging outcomes from the trial have substantially improved and will continue to inform our knowledge of the impact of AMX0035 on the neurodegenerative pathways relevant to the progression of AD, which have been and will be informative as we continue clinical development of AMX0035 in AD and other potential indications. We believe these insights will help us to examine any effects of AMX0035 on AD's progression, which could inform future work in AD as well as clinical trial design for other indications. We will continue to evaluate these data and discuss the results of the PEGASUS trial with scientific advisors as we consider potential next steps for the development of AMX0035 for the treatment of AD within our clinical development strategy.

Clinical Development of AMX0035 for Wolfram Syndrome

Wolfram syndrome is a rare, pediatric, life-threatening disease thought to be caused by variants in the Wolfram syndrome WFS1 gene, or WFS1, and, in a small fraction of patients, pathogenic

variants in the CDGSH iron sulfur domain protein 2 CISD2 gene, or CISD2. Wolfram syndrome results in deafness, blindness, ataxia, neurodegeneration and ultimately death. There are currently no drugs approved for Wolfram syndrome.

Wolfram syndrome appears to be a disease of ER stress. WFS1 encodes and produces the vital wolframin protein, which appears to be involved in ER regulatory processes. WFS1 deficiency leads to chronic ER stress and the UPR. WFS1 also negatively regulates activating transcription factor 6 (ATF6), a UPR molecule, resulting in cell death. Furthermore, a recent study suggested that WFS1 impacts mitochondrial function by transporting Ca2+ from the ER to the mitochondria through the MAM.

AMX0035 targets pathways central to Wolfram syndrome, including the UPR, and has shown beneficial effects in a variety of models of Wolfram syndrome, including cellular models and patient-derived cell line models. For example, to test the potential effects of AMX0035 in the modulation of ER stress in the context of Wolfram syndrome, the effects of PB, TURSO and AMX0035 were tested in an *in vitro* model of wild-type and WFS1-deficient pancreatic beta cell lines. In these cells, when compared with the control group, only AMX0035, but not PB or TURSO alone, was able to significantly prevent tunicamycin-induced cell death in WFS1-deficient pancreatic beta cell lines as measured by caspase 3 / 7 activity (p equal to 0.017). Additionally, a combination of PB and TURSO was studied *in vitro* in human patient-derived neural progenitor cells harboring mutations in WFS1, which cause Wolfram Syndrome. Both PB and TURSO, when applied alone, were observed to inhibit cell death in each of three different human cell lines as compared to control conditions, and the application of PB and TURSO in combination was observed to result in significantly lower levels of cell death in three separate patient-derived Wolfram syndrome cell lines differentiated to produce patient-derived neural progenitor cells, as compared to either the control or treatment with PB or TURSO alone. In each of these models of Wolfram syndrome, use of AMX0035 was observed to have significant synergistic effects lowering cell death as compared to either the control group or treatment with PB or TURSO alone. For these reasons, we believe AMX0035 is a promising clinical candidate for Wolfram syndrome and we are planning to pursue a clinical trial in this disease.

Patient Advocacy

The patient advocacy landscapes for ALS, AD and other neurodegenerative diseases are large, and encompass groups at the international, multiregional and country-specific level. We have built strong medical and commercial relationships at the international level, with our current emphasis being on ALS advocacy groups in Canada, the United States and Europe. We plan to engage country-specific groups in Europe based on clinical trial results, as well as our medical and commercial priorities.

Working with key advocacy groups is critical to our mission, as patients are at the center of everything we do. This starts with transparent communication and awareness about our science, data and development plans. We seek ensure that these advocacy groups are informed and able to answer questions from their members about PB, TURSO and AMX0035.

We engaged with patient advocacy groups in the United States and Europe for feedback on the design of our ongoing global Phase 3 PHOENIX trial of AMX0035 for the treatment of ALS, which is emblematic of the partnerships we are building with the community. In addition, we treat patient advocacy groups as important stakeholders as we address access to AMX0035 outside ongoing clinical trials, such as expanded access and compassionate use programs. We have sought and will continue to seek guidance and insights from as many patient advocacy groups as possible and have plans in place to engage groups on an ongoing basis. These groups have also reviewed messaging and press releases from the company to ensure they take into account the patient voice.

Commercialization

We believe the global commercial opportunity for AMX0035 in ALS is driven by its being the first and only treatment for ALS of which we are aware that potentially provides a combination of longer retention of function, improved survival, a generally well-tolerated side effect profile and convenient oral administration. AMX0035 has been shown to have a significant impact on clinically meaningful endpoints, including reducing time to first hospitalization and permanent ventilation in ALS patients. AMX0035 is also being considered for other neurodegenerative disorders.

ALS is a rare disease, but public sources estimate approximately 25,000 prevalence ALS patients in the United States. We believe the prevalence is closer to 30,000 in the United States based on research that we have conducted in collaboration with expert consulting groups. Approximately 40,000 ALS patients are estimated to be living with ALS in Europe and about 3,000 ALS patients are estimated to be living with ALS in Canada. In the United States, ALS is treated by neurologists at certified ALS Centers or by other neurologists. In Canada and in Europe, most ALS patients are treated at ALS Centers. The vast majority of people with ALS (over 90%) have sporadic disease, showing no clear family history. Most people who develop ALS are between the ages of 40 and 70, with an average age of 55 at the time of diagnosis. However, cases of the disease do occur in people in their twenties and thirties. People with ALS spend approximately one-third of their disease course searching for a diagnosis and, once diagnosed, there are few approved therapies available. ALS is a relentlessly progressive and highly heterogenous disease that arises from multiple mechanistic underpinnings, leading patients to experience variable onset, persistent progression, and shortened survival. The disease remains universally fatal with median survival of less than three years from symptom onset and less than two years from diagnosis.

We have conducted market research with physicians, patients, caregivers, nurses, and payors in the United States, Western Europe and Canada to understand the unmet need and potential of AMX0035 in ALS. Clinicians universally report dissatisfaction with currently approved therapies and state the need for additional options for their ALS patients. When shown a target product profile for AMX0035, the majority of ALS specialists and neurologists with whom we spoke are open to utilizing it in early-to-mid-stage patients, with some also stating the potential for use in late-stage patients.

We submitted an NDS for AMX0035 in ALS with Health Canada in the second quarter of 2021 which was accepted for review in the third quarter of 2021, based on the clinical data from the CENTAUR trial and feedback from Health Canada and submitted an NDA to the FDA in the fourth quarter of 2021. In addition to the submissions in Canada and with the FDA, we plan to submit an MAA in Europe planned in the first quarter of 2022. We also plan to discuss AMX0035 with other health authorities around the world to determine the most appropriate path forward in their respective territories. We also initiated our ongoing Phase 3 PHOENIX trial in the fourth quarter of 2021 to further support the safety and efficacy of AMX0035 for the treatment of ALS and our global regulatory efforts.

Our pre-launch activities include building awareness of and education regarding the disease severity and pathophysiology of ALS, increasing understanding of the clinical impact of a change in a patient's ALSFRS-R score, and building general awareness of our company through active participation in key neurology conferences, patient meetings, partnerships with patient advocacy groups, targeted omnichannel initiatives and payor education in each of the key territories. In addition, we intend to continue to pursue an active public relations strategy. For example, the double-blind results of our CENTAUR trial have already been published in the *New England Journal of Medicine*, while the long-term survival study results appeared in the *Journal of Muscle & Nerve*.

If AMX0035 is approved, our initial plans are to build out our commercial operations in Canada, the United States and Europe. There are 175 ALS Association certified, recognized, or affiliated centers in the United States, 17 Canadian ALS Research Network Clinics in Canada, and less than 11

ALS Centers of Excellence per country in the major EU countries, which we plan to target with a specialty key account management team. We will continue to evaluate market entry opportunities beyond these geographies either on our own or with a partner.

Competition

Overview

The biotechnology and pharmaceutical industries are characterized by rapid technological advancement, significant competition and an emphasis on proprietary products. We face potential competition from many different sources, including major and specialty pharmaceutical and biotechnology companies, academic research institutions, governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize, including AMX0035, may compete with current therapies and new therapies that may become available in the future. We believe that the key competitive factors affecting the success of any of our product candidates will include efficacy, safety profile, dosing, cost, effectiveness of promotional support and intellectual property protection.

Many of our competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, preclinical testing, clinical trials, manufacturing, and marketing than we do. Future collaborations and mergers and acquisitions may result in further resource concentration among a smaller number of competitors. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market or make our development more complicated. These competitors may also vie for a similar pool of qualified scientific and management talent, sites and patient populations for clinical trials, as well as for technologies complementary to, or necessary for, our programs.

AMX0035 for the Treatment of ALS

In the past 30 years, only two product candidates have been approved for the treatment of ALS in the United States and Canada, and only one product candidate has been approved for the treatment of ALS in Europe. These two approved drugs, riluzole (marketed under the name Rilutek) and edaravone (marketed under name Radicava in the United States and Radicut in Japan), are often used in combination. We expect that further therapies and drugs which may be approved in the future will also be used in combination with existing drugs, absent incompatibility or other barriers to combination.

There are currently no approved treatments for ALS which show both a functional and survival benefit for ALS patients. Patients with ALS are commonly treated with riluzole and edaravone, which are palliative in nature. We believe that these two drugs will not directly compete with AMX0035, if approved, as we believe that successful treatment will likely require concurrent targeting of multiple key neuron death pathways. However, we are aware of several product candidates in clinical development that may compete with AMX0035 for the treatment of ALS, if approved, including product candidates being developed by Biogen, Biohaven and UCB. To date, we believe none of the above product candidates has shown statistically significant clinical results on prespecified outcomes in any prior trials. We anticipate that ALS will continue to be an area of research in in the healthcare sector and that drug candidates will continue to be developed and studied for treatment of the disease.

While we anticipate the general practice in ALS will continue to be the combination of approved agents, our commercial potential could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more

convenient or are less expensive than products that we may develop. In addition, we are aware of one ongoing clinical study in Europe which is evaluating the effects on ALS of TURSO, one of the two components in AMX0035. The outcome of this study could have an impact on the commercial potential of AMX0035.

A large number of trials and studies are ongoing in the many additional neurodegenerative diseases which we are evaluating for future clinical work for AMX0035 including AD, Wolfram syndrome, Parkinson's Disease, Huntington's Disease, Progressive Supranuclear Palsy, Multi-System Atrophy, primary lateral sclerosis, ischemic stroke, MS, Friedreich's ataxia, Leigh's syndrome and Leber's hereditary optic neuropathy. Some of these diseases also have therapies approved which impact disease progression. The competitive landscape in these diseases will affect the potential opportunity for AMX0035.

Supply and Manufacturing

Currently, we do not have the infrastructure nor internal capability to manufacture AMX0035 for use in clinical development, and if approved, commercialization. We rely, and expect to continue to rely for the foreseeable future, on third-party contract manufacturing organizations, or CMOs, for the production of AMX0035 in compliance with current Good Manufacturing Process, or cGMP, requirements, for use in clinical trials under the guidance of members of our organization. For AMX0035, we utilize two active pharmaceutical ingredients, or APIs, PB and TURSO, which are manufactured and released to us from third-party manufacturers. We have long term, single-source supply agreements in place for these APIs, including authorization to reference the relevant drug master files with these vendors. We have single-source arrangements for the manufacturing and packaging of bulk drug at established CMOs for our clinical trials, and we expect that these CMOs will supply commercial product if AMX0035 is approved. We manufacture AMX0035 bulk drug at Patheon Inc., or Patheon, a subsidiary of Thermo Fisher Scientific Inc., located in Whitby, Canada. We have scaled-up our third-party manufacturing capabilities in a manner that we believe will support commercial demand and have entered into agreements covering the manufacture of AMX0035 through 2025. Following manufacturing, bulk drug is then sent to PCI Pharma Services in Rockford, IL, for primary and secondary packaging. As we look to markets outside of the United States, we plan to add additional manufacturing and distribution sites to support local market demand. In addition, we utilize a risk-based approach to bring on additional manufacturing sites as needed.

We have built a team of pharmaceutical industry technical operations leaders. This team has significant technical, manufacturing, analytical, quality, regulatory, including cGMP, and project management experience to oversee our third-party manufacturers and maintain quality and regulatory compliance. In addition, members of this team have been involved in commercializing and launching rare disease products across the globe. We plan to continue to build our technical operations team as we move towards commercialization.

Manufacturing Agreement with Patheon

In November 2019, we entered into a master manufacturing services agreement, or the Manufacturing Agreement, with Patheon, pursuant to which Patheon provides cGMP manufacturing, quality control, quality assurance, stability testing, packaging and related services to us. We have executed an initial product agreement under the Manufacturing Agreement, which covers AMX0035. The Manufacturing Agreement has an initial term ending in December 2025, and will automatically renew if there is a product agreement in effect, with the renewal period ending upon the termination of the last product agreement in effect. The product agreement covering AMX0035 has an initial term ending in December 2025 and automatically renews for successive terms of two years, unless either party gives prior notice of its intent not to review.

We may terminate the Manufacturing Agreement or any product agreement: upon 30 days' prior written notice if any government or regulatory authority permanently prevents us from selling AMX0035 in Canada, the EU or the United States, if approved, or upon 90 days' prior written notice, if we no longer intend to order manufacturing services due to AMX035's discontinuance in the market. Patheon may terminate any product agreement under the Manufacturing Agreement upon 30 days' prior written notice, if we project zero volume for twelve successive months during the term of such product agreement. Additionally, Patheon may terminate the Manufacturing Agreement or any product agreement if payment in full of any overdue, undisputed invoice is not received within 30 days of Patheon's suspension of manufacturing services for nonpayment or, in certain circumstances, upon nine months' prior written notice if we assign any rights under the Manufacturing Agreement or a product agreement. In addition, either party may terminate the Manufacturing Agreement or any product agreement for cause, including the other party's uncured material breach and upon written notice, in the case of the other party's insolvency or bankruptcy.

Supply Agreement with CU Chemie

In October 2019, we entered into a supply agreement with CU Chemie Uetikon, GmbH, or CU Chemie, a division of the SEQENS group, pursuant to which CU Chemie agreed to supply to us, on a non-exclusive basis, bulk drug substance of PB, for use in the manufacture of AMX0035. The agreement has an initial term of five years and will automatically renew for successive terms of two years, unless earlier terminated. After the expiration of the initial term, either party may terminate the agreement for convenience upon three months' prior written notice. Additionally, either party may terminate the agreement in the case of the other party's uncured material breach or upon the insolvency or bankruptcy of the other party.

Supply Agreement with ICE

In December 2019, we entered into a supply agreement with Prodotti Chimici e Alimentari S.p.A. (now ICE S.p.A., or ICE), as amended in July 2021, pursuant to which ICE agreed to supply to us, on a non-exclusive basis, bulk drug substance of TURSO, which we use in the manufacture of AMX0035. The agreement has an initial term of five years and will automatically renew for successive terms of five years, unless earlier terminated. ICE may terminate the agreement upon three months written notice. Additionally, either party may terminate the agreement in the case of the other party's insolvency or bankruptcy, or in case of the other party's uncured breach.

Intellectual Property

Intellectual property is of vital importance in our field and to pharmaceuticals generally. Our commercial success depends in part on our ability to obtain intellectual property that protects AMX0035 and its uses, and any future product candidates. We seek to protect and enhance proprietary technology, inventions and improvements that are commercially important to the development of our business and AMX0035, in particular, by seeking, maintaining and defending U.S. and foreign patent rights.

We are actively building our intellectual property portfolio in our therapeutic area, including around AMX0035. Our current patent portfolio as of the date of this prospectus includes three patent families. In those three families, we currently own a total of 69 issued patents and pending patent applications directed to our technologies, including AMX0035. Currently, our patent portfolio includes four issued U.S. patents, 47 issued foreign patents, four pending U.S. patent applications and 14 pending foreign patent applications. Our issued patents and pending applications cover the relative amounts of a phenylbutyrate compound and a bile acid (such as TUDCA) and some of our issued and pending claims cover the specific ratio of those two drugs.

Our earliest in time patent family relates to compositions of a bile acid and a phenylbutyrate compound (including TURSO and 4-PBA) and methods of treating neurodegenerative disease, and its associated causes at a cellular level, using those compositions. This family includes four issued U.S. patents and 47 issued foreign patents (including rights in countries in which our issued European patent was validated). The foreign jurisdictions in which we have been issued patents include Albania, Austria, Australia, Bosnia and Herzegovina, Belgium, Bulgaria, China, Croatia, Cyprus, Czech Republic, Denmark, Estonia, European Union, Finland, France, Germany, Greece, Hong Kong, Hungary, Ireland, Iceland, Italy, Japan, Lithuania, Latvia, Macao, Macedonia, Malta, Monaco, Montenegro, Netherlands, Norway, Poland, Portugal, Romania, San Marino, Serbia, Slovenia, Slovakia, South Korea, Spain, Sweden, Switzerland, Turkey, and United Kingdom. We also have patent applications pending in Australia, Canada, China, European Union, Hong Kong, Japan, South Korea, and the United States. In this family, we have composition of matter claims issued in the United States (U.S. Patent No. 11,071,742, which was issued on July 27, 2021) and Australia, and pending in applications filed in China and Hong Kong. These issued patents and others that issue from this family may first begin to expire as early as December 2033.

Our second patent family is directed to specific compositions of a phenylbutyrate compound and a bile acid (including TURSO and 4-PBA) and methods of manufacturing those compositions. We have patent applications pending in this family in the United States, Argentina and Taiwan, as well as a Patent Cooperation Treaty (PCT) application. In this family, we have composition of matter claims pending in applications filed in the United States, Argentina, and Taiwan. Although no patents have yet issued from this family, we expect the term on patents issuing from this family to extend until at least July 2040.

Our third patent family is directed to methods of treating particular symptoms of ALS and/or reducing the associated adverse events with combinations of a phenylbutyrate compound and a bile acid (including TURSO and 4-PBA). We have patent applications pending in this family in the United States and Taiwan, as well as a Patent Cooperation Treaty (PCT) application. Currently, we do not have any composition of matter claims pending in this family. Although no patents have yet issued from this family, we expect the term on patents issuing from this family to extend until at least August 2040.

We cannot be sure that patents will be granted with respect to any of our pending patent applications nor with respect to any patent applications that may be filed by us in the future. Further, we cannot be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products. Finally, we cannot be sure that our granted patents, and any future patents granted to us, will be found valid and/or enforceable following a litigation or administrative procedure.

In January 2021, Bruschettini S.r.l. and Lederer & Keller Patentanwälte Partnerschaft mbB each filed oppositions at the European Patent Office to our issued European Patent, EP2978419. At a high level, this patent claims various methods of treating neurodegenerative disease (and or the causes or conditions thereof) with a bile acid and a phenylbutyrate compound. The opponents contend that the patent should be revoked in its entirety on various grounds, including for allegedly having an insufficient disclosure and for lack of inventive step. The EPO has issued a preliminary opinion dated October 13, 2021, and a summon to attend oral proceedings (also dated October 13, 2021) that sets a date of June 2, 2022 for the oral proceedings. While we believe that this opposition lacks merit, the Opposition Board could revoke our patent in its entirety or limit the scope of our issued claims.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing of the first non-provisional application to which priority is claimed. In the United States, patent term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the United States Patent and Trademark Office in granting a patent, or may be shortened if a patent is

terminally disclaimed over an earlier-filed patent. In the United States, the term of a patent that covers an FDA-approved drug may also be eligible for a patent term extension of up to five years under the Hatch-Waxman Act, which is designed to compensate for the patent term lost during the FDA regulatory review process. The length of the patent term extension is calculated based on the length of time it takes for regulatory review. A patent term extension under the Hatch-Waxman Act cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Moreover, a patent can only be extended once, and thus, if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug. When possible, depending upon the length of clinical trials and other factors involved in the submission of an NDA, we expect to apply for patent term extensions for patents covering our product candidates and/or their methods of use.

We also rely on trademarks, trade secrets, know-how, continuing technological innovation, confidentiality agreements, and invention assignment agreements to develop and maintain our proprietary position. The confidentiality agreements are designed to protect our proprietary information and the invention assignment agreements are designed to grant us ownership of technologies that are developed for us by our employees, consultants, or other third parties. We seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in our agreements and security measures, either may be breached, and we may not have adequate remedies. In addition, our trade secrets may otherwise become known or independently discovered by competitors.

Our commercial success also depends in part on our ability to operate without infringing on the proprietary rights of others and to prevent others from infringing our proprietary rights. A comprehensive discussion on risks relating to intellectual property is provided under the section of this prospectus entitled “Risk Factors—Risks Related to Our Intellectual Property.”

We have conducted searches of the patent landscape at certain points and in certain jurisdictions with respect to AMX0035, and based on these searches and our analyses, we have not identified any issued patents that we believe are valid and could be successfully asserted to block our ability to commercialize AMX0035.

European Patent EP3016654, entitled “Tauroursodeoxycholic acid (TUDCA) for Use in the Treatment of Neurodegenerative Disorders,” is owned by Bruschettini S.r.l. The patent relates to use of TURSO in the treatment of amyotrophic lateral sclerosis in a mammal. An opposition has been filed to the grant of EP3016654 at the European Patent Office (EPO), asking the EPO to revoke EP3016654. The EPO issued a preliminary opinion on November 18, 2019 finding that at least the main claim of EP3016654 lacked novelty. Oral proceedings were held before an Opposition Division of the EPO on June 11, 2021. At the end of the oral proceedings, the Opposition Division announced the decision revoking all claims of EP3016654. A written decision has been issued; however Bruschettini can appeal the decision of the Opposition Division to the Board of Appeal.

Government Regulation

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries, including Canada and member states of the European Union, or EU, impose requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs, such as those we are developing. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of our product candidates.

U.S. Government Regulation of Drug Products

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending New Drug Applications, or NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- Completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;
- Submission to the FDA of an IND application, which must become effective before human clinical trials may begin;
- Approval by an independent IRB at each clinical site before each trial may be initiated;
- Performance of adequate and well-controlled human clinical trials in accordance with current Good Clinical Practices, or cGCP, requirements to establish the safety and efficacy of the proposed drug product for each indication;
- Submission to the FDA of an NDA, including payment of application user fees;
- A determination by the FDA within 60 days of its receipt of an NDA to accept the marketing application for review;
- Satisfactory completion of an FDA advisory committee review, if applicable;
- Satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practice, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- Satisfactory completion of FDA audits of clinical trial sites to assure compliance with cGCPs and the integrity of the clinical data; and
- FDA review and approval of the NDA.

Preclinical Studies

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as *in vitro* and animal studies to assess potential safety and efficacy. The conduct of preclinical studies is subject to federal regulations and requirements, including good laboratory practice regulations for safety/toxicology studies.

An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data and any available clinical data or literature and plans for clinical studies, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before human clinical trials may begin. Some preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related

to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to initiate.

Clinical Trials

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with cGCP requirements, which include the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it is initiated at that institution. The IRB also must review and approve the informed consent form that must be provided to each clinical trial subject or his or her legal representative, and must monitor the clinical trial until completion.

Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on their www.clinicaltrials.gov website. Information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Although sponsors are obligated to disclose the results of their clinical trials after completion, disclosure of the results can be delayed in some cases for up to two years after the date of completion of the trial. Failure to timely register a covered clinical study or to submit study results as provided for in the law can give rise to civil monetary penalties and also prevent the non-compliant party from receiving future grant funds from the federal government. The NIH's Final Rule on ClinicalTrials.gov registration and reporting requirements became effective in 2017, and both the NIH and FDA recently signaled the government's willingness to begin enforcing those requirements against non-compliant clinical trial sponsors.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase 1: The drug is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.
- Phase 2: The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- Phase 3: The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval on an NDA.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. Written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, findings from other studies or animal or in vitro testing that suggest a significant risk for human subjects and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information.

Phase 1, Phase 2 and Phase 3 trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether a trial may move forward at designated check points based on access to certain data from the trial.

Concurrent with clinical trials, companies usually complete additional animal studies and also must develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of product and, among other things, companies must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug does not undergo unacceptable deterioration over its shelf life.

Expanded Access to an Investigational Drug for Treatment Use

Expanded access, sometimes called "compassionate use," is the use of investigational products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational products for patients who may benefit from investigational therapies. FDA regulations allow access to investigational products under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the investigational product under a treatment protocol or treatment IND application.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere with initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its drug products available for expanded access; however, as required by the 21st Century Cures Act, or Cures Act, passed in 2016, if a sponsor has a policy regarding how it responds to expanded access requests, it must make that policy publicly available. Sponsors are required to make such policies publicly available upon the earlier of initiation of a Phase 2 or Phase 3 trial; or 15 days after the investigational drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under FDA expanded access program. There is no obligation for a manufacturer to make its investigational products available to eligible patients as a result of the Right to Try Act.

Combination Rule for Fixed-Dose Combination Products

Under the combination rule, the FDA may not file or approve an NDA for a fixed-dose combination product unless each component of a proposed drug product is shown to make a contribution to the claimed effects and the dosage of each component (amount, frequency, duration) is safe and effective for the intended population. To satisfy these requirements, the FDA typically requires a clinical factorial study, designed to assess the effects attributable to each drug in the combination product. This is particularly true when the ingredients are directed at the same sign or symptom of the disease or condition.

The FDA has accepted a variety of approaches to satisfy the combination rule. In December 2015, the FDA proposed regulations that would allow the agency to waive the requirements of the combination rule for certain drug products under particular circumstances. The FDA has not, to date, finalized these regulations, but the FDA has stated that factorial studies may be unethical (e.g., omitting a drug known to improve survival) or impractical (there may be too many components to conduct a factorial study, meaning the trial cannot be conducted). The FDA has also stated that it may be possible to use other types of clinical and nonclinical data and mechanistic information available to demonstrate the contributions of the individual active ingredients to the effect of the combination.

Similar requirements may be imposed on us by the EMA in the EU and comparable regulatory authorities in other jurisdictions. While no similar combination rule formally exists in Canada, Health Canada may consider the contributions of each component in a combination product in connection with review of the NDS.

NDA Submission and Marketing Approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. The FDA will initially review an NDA for completeness before it accepts it for "filing." Under the FDA's procedures, the agency has 60 days from its receipt of the NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that the application is sufficiently complete to permit substantive review. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA, for a new molecular entity to review and act on the submission, and six months from the filing date of a new molecular

entity NDA with priority review. Accordingly, this review process typically takes 12 months and eight months, respectively from the date the NDA is submitted to the FDA. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs, and the review process is often extended by FDA requests for additional information or clarification. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

In addition, under the Pediatric Research Equity Act of 2003, or PREA, as amended, certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration must submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

The FDA may refer an application for a novel drug or a drug that presents difficult questions of safety or efficacy to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA also may require the submission of a Risk Evaluation and Mitigation Strategy, or REMS, if it determines that a REMS is necessary to ensure that the benefits of the drug outweigh its risks and to assure the safe use of the drug. A REMS may include one or more elements, including medication guides, physician communication plans, patient package insert and/or elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools. The FDA determines the requirement for a REMS, as well as the specific REMS provisions, on a case-by-case basis. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS. The FDA will not approve the NDA without a REMS, if required.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with cGCP requirements.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a Complete Response Letter. A Complete Response Letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for FDA to reconsider the application. If a Complete Response Letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug product intended to treat a rare disease or condition, which is generally a disease or condition that affects either (i) fewer than 200,000 individuals in the United States, or (ii) more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making the product available in the United States for this type of disease or condition will be recovered from sales of the product. A company must request orphan drug designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product is entitled to orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications to market the same product for the same indication for seven years, except in certain limited circumstances. If a drug designated as an orphan drug ultimately receives marketing approval for an indication broader than what it was designated for, it may not be entitled to exclusivity. Orphan exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with

orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan drug has exclusivity. U.S. lawmakers have also recently raised the possibility that regulatory or legislative changes might need to be made to the Orphan Drug Act to foster competition. This includes the introduction of legislation that, if adopted into law, would require us to demonstrate to the FDA that AMX0035 would be economically unviable when facing competition to maintain our exclusivity.

Expedited Development and Priority Review Programs

The FDA maintains several programs intended to facilitate and expedite development and review of new drugs to address unmet medical needs in the treatment of serious or life-threatening diseases or conditions. These programs include Fast Track designation, Breakthrough Therapy designation, Priority Review and Accelerated Approval, and the purpose of these programs is to either expedite the development or review of important new drugs to get them to patients earlier than under standard FDA development and review procedures.

The FDA has a FastTrack program that is intended to expedite or facilitate the process for reviewing new drugs that meet certain criteria. Specifically, new drugs are eligible for Fast Track designation if they are intended to treat a serious or life threatening condition and preclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to both the product and the specific indication for which it is being studied. The sponsor can request the FDA to designate the product for Fast Track status any time before receiving NDA approval, but ideally no later than the pre-NDA meeting. Fast Track designation provides increased opportunities for sponsor interactions with the FDA during preclinical and clinical development, in addition to the potential for rolling review once a marketing application is filed, meaning that the agency may review portions of the marketing application before the sponsor submits the complete application, as well as priority review, discussed below.

Additionally, a drug may be eligible for designation as a breakthrough therapy if the product is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints. The benefits of Breakthrough Therapy designation include the same benefits as Fast Track designation, plus intensive guidance from the FDA to ensure an efficient drug development program. A product may also be eligible for priority review if it treats a serious or life-threatening condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA determines at the time that the marketing application is submitted, on a case-by-case basis, whether the proposed drug represents a significant improvement in treatment, prevention or diagnosis of disease when compared with other available therapies. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review in an effort to facilitate the review and to shorten the FDA's goal for taking action on an NDA for a new molecular entity from ten months to six months from the date of filing.

A product may also be eligible for accelerated approval if it treats a serious or life-threatening disease or condition, generally provides a meaningful advantage over available therapies and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. As a condition of accelerated

approval, the FDA requires that a sponsor perform adequate and well-controlled post-marketing clinical trials. If the FDA concludes that a drug shown to be effective can be safely used only if distribution or use is restricted, it will require such post-marketing restrictions, as it deems necessary to assure safe use of the product.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Fast Track designation, Breakthrough Therapy designation and Priority Review designation do not change the standards for approval, but may expedite the development or review process. Drugs granted accelerated approval also must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

U.S. Non-Patent Exclusivity

Data exclusivity provisions under the FDCA can delay the submission or the approval of certain follow-on applications. The FDCA provides a five-year period of data exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an Abbreviated New Drug Application, or ANDA, for a generic version of the drug or a 505(b)(2) NDA for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, such a follow-on application may be submitted after four years if it contains a certification of patent invalidity or non-infringement.

The FDA has previously taken the position that new chemical entity or NCE exclusivity is not available for fixed-dose combination products if one of the active moieties in the combination product had been previously approved in a drug product. In October 2014, however, the FDA reversed that position when it issued final guidance stating that an application for a fixed-dose combination product will be eligible for 5-year NCE exclusivity if it contains a drug substance with a single, new active moiety, even if the fixed-combination also contains a drug substance with a previously approved active moiety.

The FDCA also provides three years of market exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity period covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving follow-on applications that do not reference the protected clinical data. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of regulatory market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing regulatory exclusivity periods or listed patents. This six-month exclusivity may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued “Written Request” for such a trial.

Post-approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There are continuing, annual user fee requirements for any marketed products.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

FDA regulations require that products be manufactured in specific facilities and in accordance with cGMP regulations which require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Manufacturers and other parties involved in the drug supply chain for prescription drug products must also comply with product tracking and tracing requirements and for notifying FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the United States. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval of a drug is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Other potential consequences include, among other things:

- Restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- Fines, warning letters or holds on post-approval clinical trials;
- Refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or withdrawal of product approvals;
- Product seizure or detention, or refusal to permit the import or export of products; and
- Injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted by a manufacturer and any third parties acting on behalf of a manufacturer only for the approved indications and in a manner consistent with the

approved label for the product. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

Other U.S. Healthcare Laws

Healthcare providers, physicians, and third-party payors will play a primary role in the recommendation and prescription of drug products for which we obtain marketing approval. Arrangements with third-party payors, healthcare providers and physicians, in connection with the clinical research, sales, marketing and promotion of products, once approved, and related activities, may expose a pharmaceutical manufacturer to broadly applicable fraud and abuse and other healthcare laws and regulations. In the United States, these laws include, without limitation, state and federal anti-kickback, false claims, physician transparency, and patient data privacy and security laws and regulations, including but not limited to those described below:

- the Anti-Kickback Statute, or AKS, which makes it illegal for any person or entity, including a prescription drug manufacturer (or a party acting on its behalf) to knowingly and willfully solicit, receive, offer or pay any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, that is intended to induce or reward, referrals including the purchase, recommendation, order or prescription of a particular drug for which payment may be made under a federal healthcare program, such as the Medicare and Medicaid programs. The AKS has been interpreted to apply to arrangements between therapeutic product manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Further, courts have found that if “one purpose” of remuneration is to induce referrals, the AKS is violated. In addition, the government may assert that a claim including items or services resulting from a violation of the AKS constitutes a false or fraudulent claim for purposes of the federal False Claims Act, or FCA;
- the federal civil and criminal false claims laws, including the FCA, which can be enforced by private citizens through “qui tam” or “whistleblower” actions, and civil monetary penalty laws, which impose criminal and civil penalties against individuals or entities for, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal health care programs that are false or fraudulent; knowingly making or causing a false statement material to a false or fraudulent claim or an obligation to pay or transmit money or property to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing such an obligation. Pharmaceutical and other healthcare companies have been, and continue to be, prosecuted under these laws, among other things, for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product and for causing false claims to be submitted because of the companies’ marketing of the product for unapproved, off-label, and thus generally non-reimbursable, uses. Similar to the AKS, a person or entity does not need to have actual knowledge of these statutes or specific intent to violate them in order to have committed a violation.
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating

to healthcare matters. Like the AKS, the Patient Protection and Affordable Care Act, or the ACA, amended the intent standard for certain healthcare fraud statutes under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the creation, use, receipt, maintenance or disclosure of individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorneys' fees and costs associated with pursuing federal civil actions;
- the federal Physician Payments Sunshine Act, created under the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the Centers for Medicare and Medicaid Services, or CMS, under the Open Payments Program, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, these reporting obligations will extend to include transfers of value made during the previous year to certain non-physician providers such as physician assistants and nurse practitioners; and
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to file reports with states regarding pricing and marketing information, such as the tracking and reporting of gifts, compensations and other remuneration and items of value provided to healthcare professionals and entities; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of a pharmaceutical manufacturer's business activities could be subject to challenge under one or more of such laws. Efforts to ensure that business arrangements comply with applicable healthcare laws involve substantial costs. It is possible that governmental and enforcement authorities will conclude that a pharmaceutical manufacturer's business practices do not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against a pharmaceutical manufacturer, and it is not successful in defending itself or asserting its rights, those actions could have a significant impact on its business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, imprisonment,

monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, reporting obligations and oversight if we become subject to integrity and oversight agreements to resolve allegations of non-compliance, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of operations, any of which could adversely affect a pharmaceutical manufacturer's ability to operate its business and the results of operations. In addition, commercialization of any drug product outside the United States will also likely be subject to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state and federal health information privacy laws, and federal and state consumer protection laws, govern the collection, use, disclosure, and protection of health-related and other personal information. For example California recently enacted the California Consumer Privacy Act, or CCPA, which creates new individual privacy rights for California consumers (as defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA will require covered companies to provide certain disclosures to consumers about its data collection, use and sharing practices, and to provide affected California residents with ways to opt-out of certain sales or transfers of personal information. The CCPA went into effect on January 1, 2020, and the California State Attorney General has submitted various versions of final regulations. The California State Attorney General also now has the authority to commence enforcement actions against violators as of July 1, 2020. Further, a new California privacy law, the California Privacy Rights Act, or CPRA, was passed by California voters on November 3, 2020. The CPRA will create additional obligations with respect to processing and storing personal information that are scheduled to take effect on January 1, 2023 (with certain provisions having retroactive effect to January 1, 2022). We will continue to monitor developments related to the CPRA and anticipate additional costs and expenses associated with CPRA compliance. Other U.S. states also are considering omnibus privacy legislation (with one additional law already passed in Virginia) and industry organizations regularly adopt and advocate for new standards in these areas. While the CCPA and CPRA contain an exception for certain activities involving PHI under HIPAA, we cannot yet determine the impact the CCPA, CPRA or other such future laws, regulations and standards may have on our business, as these laws either do not yet apply to us or are not yet in effect.

In the event we decide to conduct clinical trials or continue to enroll subjects in our ongoing or future clinical trials, we may be subject to additional privacy restrictions, under state and federal law or other obligations. We also may become subject to laws in other countries, including the General Data Protection Regulation in Europe.

Current and Future U.S. Healthcare Reform Legislation

Payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs and those methods are not always specifically adapted for new technologies such as gene therapy and therapies addressing rare diseases such as those we are developing. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, was enacted, which, among other things, subjected biologic products to potential competition by lower-cost biosimilars; addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations; subjected manufacturers to new annual fees and taxes for certain branded prescription drugs; created a new Medicare Part D coverage gap

discount program, in which manufacturers must agree to offer 50% (increased to 70% pursuant to the Bipartisan Budget Act of 2018, effective as of January 1, 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; and provided incentives to programs that increase the federal government's comparative effectiveness research.

Since its enactment, there have been numerous judicial, administrative, executive, and legislative challenges to certain aspects of the ACA. On June 17, 2021, the Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Prior to the Supreme Court's decision, President Biden issued an Executive Order to initiate a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The Executive Order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is unclear how other healthcare reform measures of the Biden administrations or other efforts, if any, to challenge repeal or replace the ACA, will impact our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in 2013, and, due to subsequent legislative amendments, will remain in effect through 2030 unless additional Congressional action is taken. Pursuant to the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, as well as subsequent legislation, these reductions have been suspended from May 1, 2020 through December 31, 2021 due to the COVID-19 pandemic. The American Taxpayer Relief Act of 2012 further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. The Bipartisan Budget Act of 2018, also amended the ACA, effective January 1, 2019, by increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and closing the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole."

Canadian Review and Approval Process

In Canada, our small molecule product candidates and our research and development activities are primarily regulated by the Food and Drugs Act and the rules and regulations thereunder, which are enforced by Health Canada. Health Canada regulates, among other things, the research, development, testing, approval, manufacture, packaging, labeling, storage, recordkeeping, advertising, promotion, distribution, marketing, post-approval monitoring and import and export of pharmaceutical products. The drug approval process under Canadian laws requires licensing of manufacturing facilities, carefully controlled research and testing of products, government review and approval of experimental results prior to giving approval to sell drug products. Regulators also typically require that rigorous and specific standards such Continuing Good Manufacturing Practices, or cGMP, Good Laboratory Practices, or GLP, and Continuing Good Clinical Practices, or cGCP, are followed in the manufacture, nonclinical development and clinical development, respectively, of any drug product. The processes for obtaining regulatory approvals in Canada, along with subsequent compliance with applicable statutes and regulations, require the expenditure of substantial time and financial resources. For further information, see "Risk Factors."

The principal steps required for drug approval in Canada are as follows:

Nonclinical Safety Pharmacology and Toxicology Studies

Non-clinical studies are conducted in vitro and in animals to evaluate pharmacokinetics, metabolism and possible toxic effects to provide evidence of the safety of the drug candidate prior to its administration to humans in clinical studies and throughout development. Such studies are conducted in accordance with applicable laws and GLP.

Clinical Trials

Similar regulations apply in Canada regarding clinical trials as in the United States. In Canada, Research Ethics Boards, or REBs, are used to review and approve clinical trial plans. Clinical trials involve the administration of an investigational new drug to human subjects under the supervision of qualified investigators, in most cases a physician, in accordance with cGCP requirements, which include review and approval by REBs. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the trial procedures, the parameters to be used in monitoring safety and the efficacy criteria to be evaluated and a statistical analysis plan. Human clinical trials for new drugs are typically conducted in three sequential phases, Phase 1, Phase 2 and Phase 3, as discussed above in the context of government regulation in the United States. Similar to the FDA, Health Canada also accepts foreign clinical trial data in support of marketing applications. Additionally, the manufacture of investigational drugs for the conduct of human clinical trials is subject to cGMP requirements.

New Drug Submission

In Canada, upon successful completion of Phase 3 clinical trials or earlier stage trials if agreeable to Health Canada, the company sponsoring a new drug then assembles all the nonclinical and clinical data and other testing relating to the product's pharmacology, chemistry, manufacture, and controls, and submits it to Health Canada as part of a New Drug Submission, or NDS. The NDS is then reviewed by Health Canada for approval to market the drug.

Health Canada will not approve the product unless compliance with cGMP—a quality system regulating manufacturing—is satisfactory and the NDS contains data that provide substantial evidence that the drug is safe and effective in the indication and at the dosage studied.

The testing and approval process for an NDS requires substantial time, effort and financial resources, and may take several years to complete. This is necessary to help ensure the efficacy, safety and quality of the product. Data obtained from nonclinical and clinical testing are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. Health Canada may not grant approval of an NDS on a timely basis, or at all. In Canada, NDSs are subject to user fees and these fees are typically increased annually to reflect inflation.

Health Canada also has authority to grant conditional approval of an NDS for a serious, life-threatening or severely debilitating disease or condition for which there is promising evidence of clinical effectiveness based on the available data that the drug has the potential to provide: effective treatment, prevention or diagnosis of a disease or condition for which no drug is presently marketed in Canada or a significant increase in efficacy and/or significant decrease in risk such that the overall benefit/risk profile is improved over existing therapies, preventatives or diagnostic agents for a disease or condition that is not adequately managed by a drug marketed in Canada.

Even if Health Canada approves a product candidate, it may limit the approved indications for use of the product candidate, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms.

Health Canada may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements, notification, and regulatory authority review and approval. Further, should new safety information arise, additional testing, product labeling or regulatory notification may be required.

European Union Approval Process

The process governing approval of medicinal products in the EU generally follows the same lines as in the United States. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of an MAA and granting of a marketing authorization by these authorities before the product can be marketed and sold in the EU Following the UK's departure from the EU, a separate marketing authorization will be required in order to place medicinal products on the market in Great Britain (under the Northern Irish Protocol, the EU regulatory framework will continue to apply in Northern Ireland and centralized EU authorizations will continue to be recognized).

Clinical Trial Approval

The Clinical Trials Directive 2001/20/EC, the Directive 2005/28/EC on GCP and the related national implementing provisions of the individual Member States of the European Union, or EU Member States, govern the system for the approval of clinical trials in the EU. Under this system, an applicant must obtain prior approval from the national competent authority, or NCA, of the EU Member States in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial at a specific study site after an independent ethics committee, or EC, has issued a favorable opinion. The clinical trial application must be accompanied by, among other documents, an investigational medicinal product dossier (the Common Technical Document) with supporting information prescribed by Directive 2001/20/EC, Directive 2005/28/EC, and corresponding national laws of the EU Member States and further detailed in applicable guidance documents. Under the current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial have to be reported to the NCA and ECs of the EU Member State where they occurred.

In April 2014, the new Clinical Trials Regulation, (EU) No 536/2014, was adopted, which is set to replace the current Clinical Trials Directive. The Clinical Trials Regulation will be directly applicable in all EU Member States without the need for any national implementing legislation. It will overhaul the current system of approvals for clinical trials in the EU. The new Clinical Trials Regulation aims to simplify and streamline the approval of clinical trials in the EU.

Under the new coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial will be required to submit a single application for approval of a clinical trial to a reporting EU Member State through a centralized EU Portal. The submission procedure will be the same irrespective of whether the clinical trial is to be conducted in a single EU Member State or in more than one EU Member State.

The Clinical Trials Regulation has not yet become effective. It is expected that the new Clinical Trials Regulation will come into effect following confirmation of the full functionality of the Clinical Trials Information System, the centralized EU portal and database for clinical trials foreseen by the new Clinical Trials Regulation, through an independent audit. This is currently expected to occur in December 2021. When the Clinical Trials Regulation becomes applicable, the existing Clinical Trial Directive and national legislation put in place to implement the Directive will be repealed. Following implementation of the Clinical Trials Regulation, a transitional period will be in effect for one year where new clinical trial applications can be submitted either under the existing Clinical Trials Directive or under the new Clinical Trials Regulation.

PRIME Designation in the EU

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIority MEdicines, or PRIME, scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation where the marketing authorization application will be made through the centralized procedure. Eligible products must target conditions for which where is an unmet medical need (there is no satisfactory method of diagnosis, prevention or treatment in the EEA or, if there is, the new medicine will bring a major therapeutic advantage) and they must demonstrate the potential to address the unmet medical need by introducing new methods of therapy or improving existing ones. Products from small- and medium-sized enterprises, or SMEs, may qualify for earlier entry into the PRIME scheme than larger companies. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and accelerated MAA assessment once a dossier has been submitted. Importantly, a dedicated contact and rapporteur from the EMA's Committee for Human Medicinal Products, or CHMP, or Committee for Advanced Therapies, are appointed early in PRIME scheme facilitating increased understanding of the product at EMA's Committee level. A kick-off meeting initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies.

Fixed-Dose Combination Guideline

As with the FDA, the EMA has also issued regulations to address review and approval of fixed-dose combination products. This EMA's Guideline on clinical development of fixed combination medicinal products came into force on October 1, 2017. The basic scientific requirements for any fixed combination medicinal product are justification of the pharmacological and medical rationale for the combination, and establishment of the evidence base for the relevant contribution of all active substances to the desired therapeutic effect (efficacy and/or safety) and a positive benefit-risk for the combination in the targeted indication. For products that involve initial combination of two active ingredients, the EMA has indicated that the design of clinical efficacy/safety studies to support a fixed combination medicinal product application for initial treatment will depend on its rationale, specifically to achieve superior efficacy or improved safety compared to use of the single active substances. In situations when it has been established that monotherapy will not be adequate, appropriate or ethical to reach the desired therapeutic effect, initial use of combination therapy should be easily justified (*e.g.* HIV).

Marketing Authorization

To obtain a marketing authorization for a product European Economic Area (*i.e.*, the EU as well as Iceland, Liechtenstein and Norway), or EEA, an applicant must submit an MAA either under a centralized procedure administered by the EMA, or one of the procedures administered by competent authorities in the EU Member States (decentralized procedure, national procedure or mutual

recognition procedure). A marketing authorization may be granted only to an applicant established in the EEA. Regulation (EC) No 1901/2006 provides that prior to obtaining a marketing authorization in the EU, applicants have to demonstrate compliance with all measures included in an EMA-approved Paediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted (1) a product-specific waiver, (2) a class waiver or (3) a deferral for one or more of the measures included in the PIP (for example, when this data is not needed or appropriate because the product is likely to be ineffective or unsafe in children, the disease or condition for which the product is intended occurs only in adult populations, or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients). Products that are granted a marketing authorization with the results of the pediatric clinical trials conducted in accordance with the PIP (even where such results are negative) are eligible for six months' supplementary protection certificate extension (if any is in effect at the time of approval).

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid across the EEA. Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy medicinal products (*i.e.* gene therapy, somatic-cell therapy and tissue-engineered medicinal products)and products with a new active substance indicated for the treatment of certain diseases, including HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of public health, the centralized procedure may be optional. The centralized procedure may at the request of the applicant also be used in certain other cases. We anticipate that the centralized procedure will be mandatory for the product candidates we are developing.

Under the centralized procedure, the CHMP is responsible for conducting the initial assessment of a product and for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Clock stops may extend the timeframe of evaluation of a MAA considerably beyond 210 days. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. If the CHMP accepts such request, the time limit of 210 days will be reduced to 150 days (excluding clock stops) but it is possible that the CHMP can revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment. At the end of this period, the CHMP provides a scientific opinion on whether or not a marketing authorization should be granted in relation to a medicinal product. Where the CHMP gives a positive opinion, the EMA provides the opinion together with supporting documentation to the European Commission, who make the final decision to grant a marketing authorization, which is issued within 67 days of receipt of the EMA's recommendation.

The European Commission may grant a so-called "marketing authorization under exceptional circumstances". Such authorization is intended for products for which the applicant can demonstrate that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use, because either (i) the indications for which the product in question is intended are encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence; (ii) in the present state of scientific knowledge, comprehensive information cannot be provided; or (iii) it would be contrary to generally accepted principles of medical ethics to collect such information. Consequently,

marketing authorization under exceptional circumstances may be granted subject to certain specific obligations, which may include the following:

- the applicant must complete an identified program of studies within a time period specified by the competent authority, the results of which form the basis of a reassessment of the benefit/risk profile;
- the medicinal product in question may be supplied on medical prescription only and may in certain cases be administered only under strict medical supervision, possibly in a hospital and in the case of a radiopharmaceutical, by an authorized person; and
- the package leaflet and any medical information must draw the attention of the medical practitioner to the fact that the particulars available concerning the medicinal product in question are as yet inadequate in certain specified respects.

A marketing authorization under exceptional circumstances is subject to annual review to reassess the risk-benefit balance in an annual reassessment procedure. Continuation of the authorization is linked to the annual reassessment and a negative assessment could potentially result in the marketing authorization being suspended or revoked. The renewal of a marketing authorization of a medicinal product under exceptional circumstances, however, follows the same rules as a “normal” marketing authorization. Thus, a marketing authorization under exceptional circumstances is granted for an initial five years, after which the authorization will become valid indefinitely, unless the EMA decides that safety grounds merit one additional five-year renewal.

The EU medicines rules expressly permit the EU Member States to adopt national legislation prohibiting or restricting the sale, supply or use of any medicinal product containing, consisting of or derived from a specific type of human or animal cell, such as embryonic stem cells. While the products we have in development do not make use of embryonic stem cells, it is possible that the national laws in certain EU Member States may prohibit or restrict us from commercializing our products, even if they have been granted an EU marketing authorization.

Unlike the centralized authorization procedure, the decentralized marketing authorization procedure requires a separate application to, and leads to separate approval by, the competent authorities of each EU Member State in which the product is to be marketed. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States who, within 90 days of receipt, must decide whether to approve the assessment report and related materials. If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the European Commission, whose decision is binding on all EU Member States.

The mutual recognition procedure similarly is based on the acceptance by the competent authorities of the EU Member States of the marketing authorization of a medicinal product by the competent authorities of other EU Member States. The holder of a national marketing authorization may submit an application to the competent authority of an EU Member State requesting that this authority recognize the marketing authorization delivered by the competent authority of another EU Member State.

Conditional Marketing Authorization

The European Commission may also grant a so-called “conditional marketing authorization” prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional marketing authorizations may be granted for product candidates (including medicines designated as orphan medicinal products), if (i) the risk-benefit balance of the product candidate is positive, (ii) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data post-authorization, (iii) the product fulfills an unmet medical need and (iv) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization. A conditional marketing authorization can be converted into a standard centralized marketing authorization (no longer subject to specific obligations) once the marketing authorization holder fulfils the obligations imposed and the complete data confirm that the medicine’s benefits continue to outweigh its risks.

Regulatory Data Protection in the EU

In the EEA, innovative medicinal products approved on the basis of a complete independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. Data exclusivity, if granted, prevents applicants for authorization of generics or biosimilars of these innovative products from referencing the innovator’s preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization, for a period of eight years from the date on which the reference product was first authorized in the EEA. During an additional two-year period of market exclusivity, a generic or biosimilar MAA can be submitted and authorized, and the innovator’s data may be referenced, but no generic or biosimilar medicinal product can be placed on the EEA market until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be an innovative medical product so that the innovator gains the prescribed period of data exclusivity, another company nevertheless could also market another version of the product if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Periods of Authorization and Renewals

A marketing authorization has an initial validity for five years in principle. The marketing authorization may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA (for a centrally authorized product) or by the competent authority of the relevant EU Member State(for a centrally authorized product). To this end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide, on justified grounds relating to pharmacovigilance, to proceed with one further five-year period of marketing authorization.

Once subsequently definitively renewed, the marketing authorization shall be valid for an unlimited period. Any authorization which is not followed by the actual placing of the medicinal product on the EEA market (in case of centralized procedure) or on the market of the authorizing EU Member State within three years after authorization, or if the product is removed from the market for three consecutive years, ceases to be valid (the so-called sunset clause).

Orphan Drug Designation and Exclusivity

Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a drug can be designated as an orphan drug by the European Commission if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition and either (i) the prevalence of the condition is affecting not more than five in ten thousand persons in the EU when the application is made, or (ii) that without incentives it is unlikely that the marketing of the drug in the EU would generate sufficient return to justify the necessary investment in its development. In each case, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the drug will be of significant benefit to those affected by that condition.

Once authorized, orphan medicinal products are entitled to ten years of market exclusivity in all EU Member States and in addition a range of other benefits during the development and regulatory review process including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure and a reduction or elimination of registration and marketing authorization fees. During the period of market exclusivity, marketing authorization may only be granted for a “similar medicinal product” with the same orphan indication if: (i) the marketing authorization holder for the original orphan medicinal product consents to the authorization of the second orphan product; or (ii) the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if it is established that this product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A “similar medicinal product” is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The period of market exclusivity may, in addition, be reduced to six years if at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation because, for example, the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity.

Regulatory Requirements After a Marketing Authorization has been Obtained

Where an authorization for a medicinal product in the EU is obtained, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include:

- Compliance with the EU’s stringent pharmacovigilance or safety reporting rules must be ensured. These rules can impose post-authorization studies and additional monitoring obligations.
- The manufacturing of authorized medicinal products, for which a separate manufacturer’s license is mandatory, must also be conducted in strict compliance with the applicable EU laws, regulations and guidance, including Directive 2001/83/EC, Directive 2003/94/EC, Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice. These requirements include compliance with EU cGMP standards when manufacturing

medicinal products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the EU with the intention to import the active pharmaceutical ingredients into the EU.

- The marketing and promotion of authorized drugs, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the EU notably under Directive 2001/83EC, as amended, and are also subject to EU Member State national laws. Direct-to-consumer advertising of prescription medicines is prohibited across the EU.

General Data Protection Regulation

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EU, including personal health data, is subject to the EU General Data Protection Regulation, or GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR will be a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance.

Brexit and the Regulatory Framework in the United Kingdom

On June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the EU, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the EU on January 31, 2020. A transition period began on February 1, 2020, during which EU pharmaceutical law remained applicable to the United Kingdom, however this ended on December 31, 2020. Since the regulatory framework in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from EU Directives and Regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom, as the United Kingdom legislation now has the potential to diverge from EU legislation. It remains to be seen how Brexit will impact regulatory requirements for product candidates and products in the United Kingdom in the long-term. The MHRA has recently published detailed guidance for industry and organizations to follow from January 1, 2021 now that the transition period is over, which will be updated as the United Kingdom's regulatory position on medical products evolves over time.

Furthermore, while the Data Protection Act of 2018 in the United Kingdom that "implements" and complements the EU's GDPR, is now effective in the United Kingdom, it is still unclear whether transfer of data from the European Economic Area, or EEA, to the United Kingdom will remain lawful under GDPR. The Trade and Cooperation Agreement provides for a transitional period during which the United Kingdom will be treated like an EU Member State in relation to processing and transfers of personal data for four months from January 1, 2021. This may be extended by two further months. After such period, the United Kingdom will be a "third country" under the GDPR unless the European

Commission adopts an adequacy decision in respect of transfers of personal data to the United Kingdom. The United Kingdom has already determined that it considers all of the EU and EEA member states to be adequate for the purposes of data protection, ensuring that data flows from the United Kingdom to the EU and EEA remain unaffected.

Pricing Decisions for Approved Products

In the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, EU Member States have the option to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage health care expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on health care costs in general, particularly prescription products, has become intense.

As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU Member States, and parallel trade, i.e., arbitrage between low-priced and high-priced EU Member States, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any products, if approved in those countries.

Rest of the World Regulation

For other countries outside of Canada, the EU and the United States, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. Additionally, the clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Coverage and Reimbursement

Successful commercialization of new drug products depends in part on the extent to which reimbursement for those drug products will be available from government health administration authorities, private health insurers, and other organizations. In the United States, government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drug products they will pay for and establish reimbursement levels. The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford a drug product. Sales of drug products depend substantially, both domestically and abroad, on the extent to which the costs of drugs products are paid for by health

maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular drug products. Third-party payors are increasingly challenging the price, examining the medical necessity, and reviewing the cost-effectiveness of medical products, therapies and services, in addition to questioning their safety and efficacy. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with branded drugs and drugs administered under the supervision of a physician. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA and foreign approvals. These studies could result in delays or disadvantageous coverage for products we develop. Our product candidates may not be considered medically necessary or cost-effective. Obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our product on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. A payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on its investment in product development. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate that we successfully develop.

In many countries, the prices of drug products are subject to varying price control mechanisms as part of national health systems. In general, the prices of drug products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for drug products, but monitor and control company profits. Accordingly, in markets outside the United States, the reimbursement for drug products may be reduced compared with the United States.

In the United States, the principal decisions about reimbursement for new drug products are typically made by CMS, an agency within the HHS. CMS decides whether and to what extent a new drug product will be covered and reimbursed under Medicare, and private payors tend to follow CMS to a substantial degree. However, no uniform policy of coverage and reimbursement for drug products exists among third-party payors and coverage and reimbursement levels for drug products can differ significantly from payor to payor. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a drug product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

We cannot be sure that coverage or reimbursement will be available for any product that we commercialize and, if coverage and reimbursement are available, what the level of reimbursement will be. Coverage may also be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Future coverage and reimbursement may be subject to increased

restrictions, such as prior authorization requirements, and to changes in the rates of reimbursement for orphan drug products both in the United States and in international markets. Reimbursement may impact the demand for, or the price of, any product for which we obtain regulatory approval.

The MMA established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. While all Medicare drug plans must give at least a standard level of coverage set by Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each Part D prescription drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for drugs for which we obtain marketing approval. Any negotiated prices for any of our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the average manufacturer price, or AMP, and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although under the current state of the law these newly eligible entities (with the exception of children's hospitals) will not be eligible to receive discounted 340B pricing on orphan drugs. As the required 340B discount is determined based on average manufacturer price, or AMP, and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase. The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. The plan for the research was published in 2012 by HHS, the Agency for Healthcare Research and Quality and the NIH, and periodic reports on the status of the research and related expenditures are made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our product candidates, if any such drug or the condition that they are intended to treat are the subject of a trial. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's drug could adversely affect the sales of our product candidate. If third-party payors do not consider our drugs to be cost-effective compared to other available therapies, they may not cover our drugs after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our drugs on a profitable basis.

These laws and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Outside of the United States, the pricing of pharmaceutical products and medical devices is subject to governmental control in many countries. For example, in the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed

only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost effectiveness of a particular therapy to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. Other countries may allow companies to fix their own prices for products, but monitor and control product volumes and issue guidance to physicians to limit prescriptions. Efforts to control prices and utilization of pharmaceutical products and medical devices will likely continue as countries attempt to manage healthcare expenditures.

Legal Proceedings

We are not currently subject to any material legal proceedings.

Facilities

Our offices are located in Cambridge, Massachusetts and consist of approximately 8,850 square feet of leased office space. The lease expires in October 2026. We believe that our facilities are adequate for our current needs and that suitable additional or substitute space would be available if needed.

Employees and Human Capital

As of October 31, 2021, we had 76 full-time employees, including a total of eight employees with M.D. and/or Ph.D. degrees. Of our workforce, 34 employees are directly engaged in research and development with the rest providing administrative, business and operations support. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider the relationship with our employees to be good. We also use outside consultants and contractors with unique expertise and skills for limited engagements. As of October 31, 2021, we utilized multiple outside consultants or contractors that represented approximately 11.2 full-time equivalents to supplement our full-time workforce.

Our human capital is integral to helping us achieve our goal to end the suffering caused by neurodegenerative diseases. Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards and cash-based performance bonus awards.

MANAGEMENT

Executive Officers and Directors

The following table sets forth the name, age as of October 31, 2021, and position of each of our executive officers and directors.

Name	Age	Position
Executive Officers		
Joshua Cohen	30	Co-Chief Executive Officer and Director
Justin Klee	30	Co-Chief Executive Officer and Director
James Frates	55	Chief Financial Officer
Margaret Olinger	56	Chief Commercial Officer
Patrick D. Yeramian, M.D.	63	Chief Medical Officer
Non-Employee Directors		
George Mclean Milne Jr, Ph.D.	77	Director
Paul Fonteyne	59	Director
Felix von Coerper	48	Director
Isaac Cheng, M.D.	46	Director
Daphne Quimi	55	Director

(1) Member of audit committee
(2) Member of compensation committee
(3) Member of nominating and corporate governance committee

Executive Officers

Joshua Cohen has served as our Co-Chief Executive Officer and a member of our board of directors since January 2014. Mr. Cohen has a B.S. in Biomedical Engineering from Brown University. We believe that Mr. Cohen is qualified to serve on our board of directors because of his experience, qualifications, attributes and skills, including his familiarity with our company, as its Co-Founder, as well as his knowledge and familiarity with corporate management.

Justin Klee has served as our Co-Chief Executive Officer and a member of our board of directors since January 2014. Mr. Klee has a B.S. in Neuroscience from Brown University. We believe that Mr. Klee is qualified to serve on our board of directors because of his experience, qualifications, attributes and skills, including his familiarity with our company, as its Co-Founder, as well as his knowledge and familiarity with corporate management.

James Frates has served as our Chief Financial Officer since January 2021. Previously, Mr. Frates served as Chief Financial Officer of Alkermes plc, a biopharmaceutical company, and its predecessor organization, from September 1998 to January 2021. Mr. Frates has an A.B. in Political Science and Government from Harvard College and an M.B.A. from the Harvard Graduate School of Business Administration. Mr. Frates serves as a member of the board of directors of Sage Therapeutics, Inc., a biopharmaceutical company.

Margaret Olinger has served as our Chief Commercial Officer since May 2019. Previously, Ms. Olinger served in various leadership and commercial positions for more than a decade at Alexion Pharmaceuticals, a biopharmaceutical company. Ms. Olinger has a B.S. in Business Administration from Albertus Magnus College and an M.B.A. from New Haven University.

Patrick D. Yeramian, M.D. has served as our Chief Medical Officer since March 2019. Dr. Yeramian has over 25 years of experience in the pharmaceutical industry has extensive leadership

experience in both early and late-stage clinical development at several innovative biopharmaceutical companies. Most recently, Dr Yeramian was the Chief Medical Officer for Tapimmune Inc., an immune-oncology company, from February 2015 to January 2017 and for Biovie Inc., a biotechnology company developing innovative drug therapies for liver disease, from October 2016 to March 2019. Earlier, from 2011 to 2015, he was the Medical Director for the Vaccine and Gene Therapy Institute of Florida. Dr. Yeramian received his M.D. from the University of Paris, as well as an M.Sc. in Experimental Oncology and a graduate degree in Molecular Virology. He also earned an M.B.A. from Rutgers University. He completed his medical residency in oncology at the Saint-Louis Hospital in Paris.

Non-Employee Directors

George Mclean Milne Jr, Ph.D. has served on our board of directors since 2015. Dr. Milne has served as a Venture Partner of Radius Ventures, LLC, a venture investment firm specializing in in entrepreneurs and companies transforming healthcare, since January 2003. Dr. Milne has served on the board of directors of Charles River Laboratories International, Inc., a laboratory services company, since May 2002. Dr. Milne had also served on the board of directors of Mettler-Toledo, Inc., an instrument manufacturing company, from June 1999 to May 2016. Dr. Milne has a B.S. in Chemistry from Yale University and a Ph.D. in Organic Chemistry from the Massachusetts Institute of Technology. We believe that Dr. Milne is qualified to serve on our board of directors because of his experience, qualifications, attributes and skills, including his more than 30 years of experience in senior executive management roles with large, international businesses.

Paul Fonteyne has served as a member of our board of directors since March 2021. Mr. Fonteyne is the retired chairman and CEO of Boehringer-Ingelheim, or BI, USA. He was with BI or BI subsidiaries from 2003 to December 2018 and made substantial contributions to BI USA. Prior to 2003, Mr. Fonteyne served in leadership positions at Merck and Co. Inc. as well as Abbot Laboratories. From December 2017 until its reverse merger with Adicet Bio in September 2020, Mr. Fonteyne served as a member of the board of directors of ResTORbio Inc., a biotechnology company and was chair of its Compensation Committee. Mr. Fonteyne has also served on the board of PhRMA, chaired the National Pharmaceutical Council and is actively participating as a founder in biopharma spinouts from Yale University in the field of Alzheimer's disease and Pulmonary disease. Mr. Fonteyne received his M.B.A. from Carnegie-Mellon University and his M.S. in Chemical Engineering from the Polytechnic School at the University of Brussels. We believe Mr. Fonteyne is qualified to serve on our board of directors because of his experience, qualifications, attributes and skills, including his past experience in the life sciences industry.

Felix von Coerper has served on our board of directors since March 2016. Mr. von Coerper is a Managing Partner at the ALS Investment Fund, a venture capital fund that invests in biotech companies developing drugs in neurodegeneration, with a particular focus in ALS. He has held this role since January 2015. Mr. von Coerper has a Masters in Economics from Erasmus University Rotterdam. We believe Mr. von Coerper is qualified to serve on our board of directors due to his financial expertise, experience as a venture capitalist and industry experience.

Isaac Cheng, M.D. has served as a member of our board of directors since March 2016. Dr. Cheng is an investment professional at the Morningside Technology Advisory, LLC, a group that invests in venture capital and private equity opportunities. He has served in this role since 2006. Dr. Cheng served on the board of directors of Atea Pharmaceuticals, Inc., a biopharmaceutical company, from March 2019 to April 2021. Dr. Cheng also served on the board of directors of NuCana PLC, a biopharmaceutical company, from May 2017 to March 2020 and Liquidia Technologies, Inc., a biotechnology company, from January 2010 to July 2018. Dr. Cheng received his M.D. and B.S. from the Tufts University School of Medicine. We believe Dr. Cheng is qualified to serve on our board of

directors due to his financial expertise, experience as a venture capitalist, industry experience and his experience in serving on the board of directors of public and private life sciences companies.

Daphne Quimi has served as a member of our board of directors since June 2021. Ms. Quimi has more than 25 years of executive experience in the pharmaceutical and biotechnology industries with expertise in global finance operations, company building, and rare disease drug commercialization. She currently serves as Chief Financial Officer of Amicus Therapeutics, Inc. ("Amicus"), a biotechnology company. Ms. Quimi has served in this role since January 2019, after holding various roles at Amicus since 2007. Prior to that, Ms. Quimi served as Director of Consolidations and External Reporting at Bristol-Myers Squibb Company, a global biopharmaceutical company, from 2005 to 2007. Ms. Quimi received a B.S. in Accountancy from Monmouth University and an M.B.A. from the Stern School of Business of New York University. We believe Ms. Quimi is qualified to serve on our board of directors due to her financial expertise and industry experience.

Board Composition and Election of Directors

Board Composition

Our board of directors currently consists of seven members, each of whom are members pursuant to the board composition provisions of our certificate of incorporation and agreements with our stockholders. These board composition provisions will terminate upon the completion of this offering. Upon the termination of these provisions, there will be no further contractual obligations regarding the election of our directors, which are described in "Certain Relationships and Related Party Transactions."

Our amended and restated certificate of incorporation and bylaws that will become effective as of the closing date of this offering provide that the authorized number of directors may be changed only by resolution of our board of directors. Our certificate of incorporation and bylaws will also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least % of our shares of capital stock present in person or by proxy and entitled to vote, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

We have no formal policy regarding board diversity. Our priority in selection of board members is identification of members who will further the interests of our stockholders through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business and understanding of the competitive landscape.

Staggered Board

In accordance with the terms of our certificate of incorporation and bylaws that will become effective as of the closing date of this offering, our board of directors will be divided into three classes, Class I, Class II and Class III, with members of each class serving staggered three-year terms. Upon the closing of this offering, the members of the classes will be divided as follows:

- the Class I directors will be and , and their term will expire at the annual meeting of stockholders to be held in 2022;
- the Class II directors will be and , and their term will expire at the annual meeting of stockholders to be held in 2023; and
- the Class III directors will be and , and their term will expire at the annual meeting of stockholders to be held in 2024.

Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires. Our amended and restated certificate of incorporation and amended and restated bylaws, both of which will become effective immediately prior to the completion of the offering provide that the number of our directors shall be fixed from time to time by a resolution of the majority of our board of directors.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control.

Director Independence

Applicable Nasdaq Global Market rules require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the Nasdaq Global Market rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. Under applicable Nasdaq Global Market rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: the source of compensation of the director, including any consulting, advisory or other compensatory fee paid by such company to the director; and whether the director is affiliated with the company or any of its subsidiaries or affiliates.

In 2021, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of is an "independent director" as defined under applicable Nasdaq Global Market rules, including, in the case of all the members of our audit committee, the independence criteria set forth in Rule 10A-3 under the Exchange Act, and in the case of all the members of our compensation committee, the independence criteria set forth in Rule 10C-1 under the Exchange Act. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director. Mr. Cohen and Mr. Klee are not independent directors under these rules because they are our Co-Chief Executive Officers.

There are no family relationships among any of our directors or executive officers.

We intend to adopt a policy, subject to and effective upon the effectiveness of the registration statement of which this prospectus forms a part, that outlines a process for our securityholders to send communications to the board of directors.

Board Leadership Structure and Board's Role in Risk Oversight

Our board of directors is currently chaired by . Our corporate governance guidelines provide that, if the Chairman of the board of directors is a member of management or does not otherwise qualify as independent, the independent directors of the board may or may not elect a lead independent director. Our corporate governance guidelines further provide the flexibility for our board of directors to modify our leadership structure in the future, as it deems appropriate.

Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks relating to our financial condition, development and commercialization activities, operations, strategic direction and intellectual property as more fully discussed under "Risk Factors" in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

The role of the board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees below and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on us, and the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairman of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables the board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

Board Committees

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee. Each of these committees will operate under a charter that has been approved by our board of directors. The composition of each committee will be effective as of the date of this prospectus.

Audit Committee

The members of our audit committee are , , and , and is the chair of the audit committee. Effective as of the date of this prospectus, our audit committee's responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- pre-approving auditing and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the overall audit plan with our independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;

- coordinating the oversight and reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending based upon the audit committee's review and discussions with management and our independent registered public accounting firm whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by SEC rules to be included in our annual proxy statement;
- reviewing all related person transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Our board of directors has determined that is an "audit committee financial expert" as defined in applicable SEC rules and that each of the members of our audit committee possesses the financial sophistication required for audit committee members under Nasdaq Global Market rules. Under the applicable Nasdaq Global Market rules, a company listed in connection with its initial public offering is permitted to phase in its compliance with the independent audit committee requirements set forth in Nasdaq Global Market rules on the same schedule as it is permitted to phase in its compliance with the independence audit committee requirement pursuant to Rule 10A-3(b)(1)(iv)(A) under the Exchange Act, that is, (1) one independent member at the time of listing; (2) a majority of independent members within 90 days of listing; and (3) all independent members within one year of listing.

Compensation Committee

The members of our compensation committee are , , and , and is the chair of the compensation committee. Effective as of the date of this prospectus, our compensation committee's responsibilities will include:

- annually reviewing and approving corporate goals and objectives relevant to the compensation of our Co-Chief Executive Officers;
- evaluating the performance of our Co-Chief Executive Officers in light of such corporate goals and objectives and determining the compensation of our Co-Chief Executive Officers;
- reviewing and approving the compensation of our other executive officers;
- reviewing and establishing our overall management compensation, philosophy and policy;
- overseeing and administering our compensation and similar plans;
- evaluating and assessing potential and current compensation advisors in accordance with the independence standards identified in the applicable Nasdaq Global Market rules;
- retaining and approving the compensation of any compensation advisors;
- reviewing and making recommendations to our board of directors about our policies and procedures for the grant of equity-based awards;

- evaluating and making recommendations to the board of directors about director compensation;
- preparing the compensation committee report required by SEC rules, if and when required, to be included in our annual proxy statement; and
- reviewing and approving the retention or termination of any consulting firm or outside advisor to assist in the evaluation of compensation matters.

Nominating and Corporate Governance Committee

The members of our nominating and corporate governance committee are , and , , and is the chair of the nominating and corporate governance committee. Effective as of the date of this prospectus, our nominating and corporate governance committee's responsibilities will include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- reviewing the size and composition of the board of directors to ensure that it is composed of members containing the appropriate skills and expertise to advise us;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board's committees;
- developing and recommending to the board of directors a code of business conduct and ethics and a set of corporate governance guidelines; and
- overseeing the evaluation of our board of directors and management.

Compensation Committee Interlocks and Insider Participation

None of our executive officers serves, or in the past year has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our board of directors or our compensation committee. None of the members of our compensation committee is, or has ever been, an officer or employee of our company.

Code of Business Conduct and Ethics

We plan to adopt a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions, which will be effective upon the closing of this offering. Following this offering, we will post a copy of the code on the Corporate Governance section of our website. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a Current Report on Form 8-K.

EXECUTIVE COMPENSATION

This section describes the material elements of compensation awarded to, earned by or paid to each of our named executive officers for the year ended December 31, 2020. We are an "emerging growth company," within the meaning of the JOBS Act, and have elected to comply with the reduced compensation disclosure requirements available to emerging growth companies under the JOBS Act. This section also provides qualitative information regarding the manner and context in which compensation is awarded to and earned by our named executive officers and is intended to place in perspective the data presented in the tables and narrative that follow.

Overview

The following discussion contains forward-looking statements that are based on our current plans and expectations regarding our future compensation programs. The actual amount and form of compensation that we pay and the compensation policies and practices that we adopt in the future may differ materially from the currently-planned programs that are summarized in this discussion.

The compensation provided to our named executive officers for the fiscal year ended December 31, 2020 is detailed in the 2020 Summary Compensation Table and accompanying footnotes and narrative that follow.

Our named executive officers for the fiscal year ended December 31, 2020, which consisted of our Co-Chief Executive Officers, our two most highly-compensated executive officers other than our Chief Executive Officer, and our former Chief Financial Officer, were:

1. Joshua B. Cohen, our Co-Chief Executive Officer and Director;
2. Justin Klee, our Co-Chief Executive Officer and Director;
3. Margaret Olinger, MBA, our Chief Commercial Officer;
4. Patrick D. Yeramian, M.D., MBA, our Chief Medical Officer; and
5. Jeffrey Trigilio, our former Chief Financial Officer.

2020 Summary Compensation Table

The following table provides information regarding the total compensation awarded to, earned by, and paid to our named executive officers for services rendered to us in all capacities for the fiscal year ended December 31, 2020.

Name and Principal Position	Year	Salary ($)	Bonus ($)	Option Awards ($)(1)	Non-Equity Incentive Plan Compensation ($) (2)	All Other Compensation ($)	Total ($)
Joshua B. Cohen *Co-Chief Executive Officer and Director*	2020	370,000	—	97,000	203,500	—	670,500
Justin Klee *Co-Chief Executive Officer and Director*	2020	370,000	—	97,000	203,500	—	670,500
Margaret Olinger, MBA *Chief Commercial Officer*	2020	360,200	—	—	154,836	—	515,036
Patrick D. Yeramian, M.D., MBA *Chief Medical Officer*	2020	373,500	—	—	124,336	—	497,836
Jeffrey Trigilio (3) *Former Chief Financial Officer*	2020	166,513	280,000	—	—	88,974	535,487

(1) The amounts reported represent the aggregate grant date fair value of the stock options awarded to the named executive officers during fiscal year 2020, calculated in accordance with Financial Accounting Standards Board, or FASB Accounting Standards Codification 718, or ASC Topic 718, *Compensation—Stock Compensation*. Such grant date fair value does not take into account any estimated forfeitures. The assumptions used in calculating the grant date fair value of the awards reported in this column are set forth in the notes to our consolidated financial statements included elsewhere in this prospectus. The amounts reported in this column reflect the accounting cost for the stock options and does not correspond to the actual economic value that may be received upon exercise of the stock option or any sale of any of the underlying shares of common stock.

(2) The amounts represent bonuses earned in 2020, and paid in February 2021, based on the achievement of pre-established performance goals as determined by our board of directors.

(3) Mr. Trigilio served as our Chief Financial Officer from January 20, 2020 until his employment terminated on July 14, 2020. The amount reported in the "Bonus" column represents a $280,000 signing bonus paid to Mr. Trigilio pursuant to his employment agreement, $80,000 of which was paid when Mr. Trigilio commenced his employment with us (the "First Payment") and $200,000 of which was paid in January 2021 (the "Second Payment"). Pursuant to the terms of his employment agreement, Mr. Trigilio was obligated to repay the First Payment in the event that he terminated his employment with us prior to January 20, 2021 but, following the termination of his employment in July 2020, we decided not to seek such repayment. The amount reported in the "All Other Compensation" column represents severance paid to Mr. Trigilio in connection with the termination of his employment.

Narrative to Summary Compensation Table

Base Salaries

Each named executive officer's base salary is a fixed component of annual compensation for performing specific duties and functions, and has been established by our board of directors taking into account each individual's role, responsibilities, skills, and expertise. Base salaries are reviewed annually, typically in connection with our annual performance review process, approved by our board of directors, and adjusted from time to time to realign salaries with market levels after taking into account individual responsibilities, performance, and experience. For fiscal year 2020, the annual base salary for (i) Mr. Cohen was $370,000, (ii) Mr. Klee was $370,000, (iii) Ms. Olinger was $360,200, (iv) Dr. Yeramian was $373,500, and (v) Mr. Trigilio was $340,000.

Annual Bonuses

For the fiscal year ended December 31, 2020, each named executive officer was eligible to earn an annual cash bonus based on the achievement of corporate performance metrics and, with respect to Ms. Olinger, Dr. Yeramian and Mr. Trigilio, on the achievement of corporate and individual performance metrics as determined by the board of directors. The 2020 target annual bonus, as a percentage of base salary, for Mr. Cohen, Mr. Klee, Ms. Olinger, Dr. Yeramian and Mr. Trigilio was 50%, 50%, 40%, 30%, and 30%, respectively.

Equity Compensation

Although we do not yet have a formal policy with respect to the grant of equity incentive awards to our executive officers, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants promote executive retention because they incentivize our executive officers to remain in our employment during the vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our named executive officers and may grant equity incentive awards to them from time to time.

In February 2020, Mr. Cohen and Mr. Klee each received options to purchase 100,000 shares of common stock, vesting over four years subject to a one year cliff and continued service.

Employment Arrangements with our Named Executive Officers

We have entered into an offer letter with each of the named executive officers in connection with their employment with us, which set forth the terms and conditions of their respective employment.

Employment Arrangements in Place During the Fiscal Year Ended December 31, 2020 for Our Named Executive Officers

Joshua B. Cohen

On July 1, 2015, we entered into an employment agreement with Mr. Cohen, who currently serves as our Co-Chief Executive Officer. In April 2021, we amended the employment agreement with Mr. Cohen effective February 19, 2021. The employment agreement provides for Mr. Cohen's at-will employment and an annual base salary, cash bonus, a stock option bonus, as well as his ability to participate in our employee benefit plans generally. Mr. Cohen's employment agreement provides that if his employment is terminated by us without "just cause" (as defined in Mr. Cohen's employment agreement), or he resigns for "good reason" (as defined in Mr. Cohen's employment agreement), Mr. Cohen will be entitled to receive (i) continuation of his then-current base salary for twelve (12) months, (ii) any cash bonus earned for a prior year that has not been paid, (iii) a pro-rata share of any bonus for which he is or becomes eligible, or "pro rata bonus", (iv) continuation of health benefits or payments equal to the company portion of health insurance premiums for up to 12 months, and (v) continued vesting of stock options for twelve (12) months. If such termination occurs less than three (3) months prior to or twelve (12) months following a "change of control" (as defined in Mr. Cohen's employment agreement) the aforementioned severance benefits shall apply except that no pro rata bonus shall be paid and any unvested stock options held by Mr. Cohen shall become fully vested and exercisable as of immediately prior to such change in control.

Justin Klee

On July 1, 2015, we entered into an employment agreement with Mr. Klee, who currently serves as our Co-Chief Executive Officer. In April 2021, we amended the employment agreement with Mr. Klee effective February 19, 2021. The employment agreement provides for Mr. Klee's at-will employment and an annual base salary, cash bonus, a stock option bonus, as well as his ability to participate in our employee benefit plans generally. Mr. Klee's employment agreement provides that if his employment is terminated by us without "just cause" (as defined in Mr. Klee's employment agreement), or he resigns for "good reason" (as defined in Mr. Klee's employment agreement), Mr. Klee will be entitled to receive (i) continuation of his then-current base salary for twelve (12) months, (ii) any cash bonus earned for a prior year that has not been paid, (iii) a pro rata bonus, (iv) continuation of health benefits or payments equal to the company portion of health insurance premiums for up to 12 months, and (v) continued vesting of stock options for twelve (12) months. If such termination occurs less than three (3) months prior to or twelve (12) months following a "change of control" (as defined in Mr. Klee's employment agreement) the aforementioned severance benefits shall apply except that no pro rata bonus shall be paid and any unvested stock options held by Mr. Cohen shall become fully vested and exercisable as of immediately prior to such change in control.

Margaret Olinger, MBA

On May 13, 2019, we entered into an employment agreement with Ms. Olinger, who currently serves as our Chief Commercial Officer. We amended the employment agreement with Ms. Olinger in

August 2019 and April 2021, the latter amendment effective as of February 19, 2021. The employment agreement provides for Ms. Olinger's at-will employment and an annual base salary, annual cash bonus, a stock option bonus, an initial stock option grant as well as her ability to participate in our employee benefit plans generally. Ms. Olinger employment agreement provides that if her employment is terminated by us without "just cause" or if she resigns for "good reason" (as such terms are defined in Ms. Olinger's employment agreement), Ms. Olinger will be entitled to receive (i) continuation of her then-current base salary for six (6) months, and (ii) reimbursement of the company portion of health insurance premiums for up to six (6) months. In addition, if such termination or resignation occurs less than three (3) months prior to or twelve (12) months following a "change of control" (as defined in Ms. Olinger's employment agreement) she shall be entitled to 100% acceleration of unvested stock options as of immediately prior to such change in control.

Patrick D. Yeramian, MD, MBA

On March 18, 2019, we entered into an employment agreement with Dr. Yeramian, who currently serves as our Chief Medical Officer. We amended the employment agreement with Dr. Yeramian in November 2019 and April 2021, the latter amendment effective as of February 19, 2021. The employment agreement provides for Dr. Yeramian's at-will employment and an annual base salary, annual cash bonus, a stock option bonus, an initial stock option grant as well as his ability to participate in our employee benefit plans generally. Dr. Yeramian's employment agreement provides that if his employment is terminated by us without "just cause" or if he resigns for "good reason" and such termination or resignation occurs less than three (3) months prior to or twelve (12) months following a "change of control" (as such terms are defined in Dr. Yeramian's employment agreement), Dr. Yeramian will be entitled to receive (i) continuation of his then-current base salary for six (6) months, (ii) reimbursement of the company portion of health insurance premiums for up to six (6) months, and (iii) 100% acceleration of unvested stock options as of immediately prior to such change in control.

Jeffrey Trigilio

On January 20, 2020, we entered into an employment agreement with Mr. Trigilio, who served as our Chief Financial Officer from January 2020 until his employment terminated in July 2020. The employment agreement provided for Mr. Trigilio's at-will employment and an annual base salary, a $280,000 sign-on bonus, annual cash bonus, stock option bonus, as well as his ability to participate in our employee benefit plans generally. Pursuant to Mr. Trigilio's employment agreement, his sign-on bonus was payable in two installments, with the First Payment of $80,000 payable within 30 days of the effective date of his employment agreement and the Second Payment of $200,000 payable on the first anniversary of the effective date. Mr. Trigilio's employment agreement provided that if he terminated his employment for any reason prior to January 20, 2021 he would be responsible for repaying 100% of the First Payment. In addition, if Mr. Trigilio were terminated by us within the first twelve (12) months for "just cause" (as defined in Mr. Trigilio's employment agreement) Mr. Trigilio would be responsible for repaying 100% of the Second Payment, assuming he had received the Second Payment. Mr. Trigilio's employment with us terminated in July 2020. In connection with such termination, we entered into a separation agreement and release with Mr. Trigilio pursuant to which Mr. Trigilio received three (3) months of severance pay equal to $28,333.33 per month and medical and dental premiums in the amount of $1,324.55 per month. Following the termination of his employment, we determined not to seek repayment of the First Payment and paid the Second Payment in January 2021 pursuant to the terms of Mr. Trigilio's employment agreement.

In connection with this offering, we intend to enter into amended and restated employment agreements with our named executive officers that will become effective upon the closing of this offering.

Outstanding Equity Awards at Fiscal 2020 Year-End

The following table sets forth information regarding outstanding equity awards held by our named executive officers as of December 31, 2020:

		Option Awards (1)			
Name	Grant Date	Number of Securities Underlying Options (#)Exercisable	Number of Securities Underlying Options (#)Unexercisable	Option Exercise Price ($)	Option Expiration Date
Joshua B. Cohen	2/16/2018(2)	236,954	97,566	0.37	2/16/2023
	2/26/2020(3)	—	100,000	1.57	2/26/2025
Justin Klee	2/16/2018(2)	236,954	97,566	0.37	2/16/2023
	2/26/2020(3)	—	100,000	1.57	2/26/2025
Margaret Olinger	5/13/2019(4)	65,371	70,209	0.37	5/13/2029
	5/13/2019(5)	58,108	77,472	0.37	5/13/2029
Patrick D. Yeramian	2/16/2018(6)	18,977	7,812	0.33	2/16/2028
	3/18/2019(7)	63,829	59,425	0.37	3/18/2029
	3/18/2019(8)	46,221	77,032	0.37	3/18/2029
Jeffrey Trigilio	—	—	—	—	—

(1) All stock options have been granted pursuant to the terms of our 2015 Stock Option and Restricted Stock Plan, as amended. Pursuant to their respective employment agreements, in the event that Mr. Klee or Mr. Cohen are terminated without just cause or resign for good reason, their stock options will continue to vest for 12 months following such termination, and in the event that such termination occurs less than three months before or 12 months following a change of control of our company, any unvested shares subject to their stock options will fully accelerate. Pursuant to their respective employment agreements, in the event that Ms. Olinger or Dr. Yeramian are terminated without just cause or resign for good reason less than three months before or 12 months following a change of control of our company, any unvested shares subject to their stock options will fully accelerate.

(2) 6,977 shares subject to this stock option vested on March 16, 2018 and the remainder is scheduled to vest thereafter in 47 monthly installments of 6,969 shares.

(3) 25,012 shares subject to this stock option vested on February 26, 2021 and the remainder is scheduled to vest thereafter in 36 monthly installments of 2,083 shares.

(4) 33,898 shares subject to this stock option vested on November 13, 2019 and the remainder is scheduled to vest thereafter in 36 monthly installments of 2,421 shares.

(5) 33,898 shares subject to this stock option vested on February 1, 2020 and the remainder is scheduled to vest thereafter in 36 monthly installments of 2,421 shares.

(6) 563 shares subject to this stock option vested on March 16, 2018 and the remainder is scheduled to vest thereafter in 47 monthly installments of 558 shares.

(7) 30,814 shares subject to this stock option vested on September 18, 2019 and the remainder is scheduled to vest thereafter in 42 monthly installments of 2,201 shares.

(8) 30,814 shares subject to this stock option vested on May 26, 2020. The remainder is scheduled to vest thereafter in 42 monthly installments of 2,201 shares.

Employee Benefits and Equity Compensation Plans

2015 Stock Option and Restricted Stock Plan

Our board of directors adopted, and our stockholders approved our 2015 Plan in April 2015. The 2015 Plan was most recently amended by our board of directors in February 2021. Under the 2015

Plan, 8,474,374 shares of our common stock have been reserved for issuance in the form of incentive stock options, non-qualified stock options, and restricted stock. The shares issuable pursuant to awards granted under the 2015 Plan are authorized but unissued shares.

The 2015 Plan is administered by our board of directors or a committee designated by our board of directors, or the "administrator," whose construction and interpretation of the terms and provisions of the 2015 Plan shall be final and conclusive. The administrator has full power to select the individuals to whom awards will be granted and to determine the specific terms and conditions of each award, subject to the provisions of the 2015 Plan.

The option exercise price of each option granted under the 2015 Plan is determined by the administrator and may not be less than the fair market value of a share of common stock on the date of grant. The term of each option is fixed by the administrator and may not exceed 10 years from the date of grant. The administrator determines at what time or times each option may be exercised when granting the option.

The 2015 Plan provides that upon the occurrence of an "acquisition event" (as defined in the 2015 Plan), the administrator shall take any one or more or none of the following actions with respect to any outstanding options: provide for the assumption or substitution of such awards by the acquiring or succeeding corporation or its affiliate; upon written notice to optionees, provide that all outstanding options will become fully exercisable as of a specified time prior to the acquisition event and will terminate immediately prior to the consummation of such acquisition event if not exercised; in the event of a merger where the holders of the Company's common stock will receive a cash or stock payment for each share surrendered in the merger, make or provide for cash or stock payment to holders of options equal to the difference between (i) the per share cash or stock consideration in the acquisition event multiplied by the number of shares subject to outstanding options that the holders elect to exercise, and (ii) the aggregate exercise price of all such outstanding options that the holders actually exercise in exchange for the termination of all such outstanding options; provide that all or any outstanding options shall become fully exercisable as of immediately prior to such acquisition event; or provide for a combination of any one or more of the foregoing options or any other plan which would be equitable, in the good faith judgment of the administrator to the holders of outstanding options.

The administrator may amend the 2015 Plan but no such action may adversely affect the rights of an award holder without such holder's consent. Approval by our stockholders of amendments to the 2015 Plan must be obtained if required by law.

As of October 31, 2021, options to purchase 4,178,341 shares of common stock were outstanding under the 2015 Plan. Our board of directors has determined not to make any further awards under the 2015 Plan following the completion of this offering.

2022 Stock Option and Incentive Plan

In connection with this offering, our board of directors, upon the recommendation of the compensation committee of the board of directors, or the compensation committee, is expected to adopt the 2022 Stock Plan, which will be subsequently approved by our stockholders. The 2022 Stock Plan is expected to become effective on the date immediately prior to the date on which the registration statement of which this prospectus is part is declared effective by the SEC. The 2022 Stock Plan is expected to replace our 2015 Plan, as our board of directors has determined not to make additional awards under the 2015 Plan following the closing of this offering. The 2022 Stock Plan will provide flexibility to our compensation committee to use various equity-based incentive awards as compensation tools to motivate our workforce.

We initially reserved shares of our common stock, or the Initial Limit, for the issuance of awards under the 2022 Stock Plan. The 2022 Stock Plan will provide that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2022, by % of the outstanding number of shares of our common stock on the immediately preceding December 31, or such lesser number of shares as determined by our compensation committee, or the Annual Increase. This number will be subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2022 Stock Plan will be authorized but unissued shares or shares that we reacquire. The shares of common stock underlying any awards that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without any issuance of stock, expire or are otherwise terminated (other than by exercise) under the 2022 Stock Plan will be added back to the shares of common stock available for issuance under the 2022 Stock Plan.

The maximum aggregate number of shares that may be issued in the form of incentive stock options may not exceed the Initial Limit cumulatively increased on January 1, 2023, and on each January 1 thereafter by the lesser of the Annual Increase for such year or shares of common stock.

The grant date fair value of all awards made under our 2022 Stock Plan and all other cash compensation paid by us to any non-employee director in any calendar year may not exceed for the first year of service and for each year of service thereafter.

The grant date fair value of all awards made under our 2022 Plan and all other cash compensation paid by us to any non-employee director in any calendar year for services as a non-employee director shall not exceed $; provided, however, that such amount shall be $ for the calendar year in which the applicable non-employee director is initially elected or appointed to the board of directors.

The 2022 Stock Plan will be administered by our compensation committee. Our compensation committee will have full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, to make any combination of awards to participants, and to determine the specific terms and conditions of each award, subject to the provisions of the 2022 Stock Plan. Persons eligible to participate in the 2022 Stock Plan will be those full or part-time employees, non-employee directors and consultants of us and our affiliates, as selected from time to time by our compensation committee in its discretion.

The 2022 Stock Plan will permit the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code and options that do not so qualify. The option exercise price of each option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each option will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each option may be exercised.

Our compensation committee may award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price of each stock appreciation right may not be less than 100% of the fair market value of the common stock on the date of grant. The term of each stock appreciation right will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each stock appreciation right may be exercised.

Our compensation committee will be permitted to award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment or service relationship with us through a specified vesting period. Our compensation committee will also be permitted to grant shares of common stock that are free from any restrictions under the 2022 Stock Plan. Unrestricted stock may be granted to participants in recognition of past services or other valid consideration and may be issued in lieu of cash compensation due to such participant.

Our compensation committee will be permitted to grant cash bonuses under the 2022 Stock Plan to participants, subject to the achievement of certain performance goals.

The 2022 Stock Plan will provide that upon the effectiveness of a "sale event," as defined in the 2022 Stock Plan, an acquirer or successor entity may assume, continue or substitute outstanding awards under the 2022 Stock Plan. To the extent that awards granted under our 2022 Stock Plan are not assumed or continued or substituted by the successor entity, except as may be otherwise provided in the relevant award certificate, all awards with time-based vesting, conditions or restrictions will become fully vested and nonforfeitable as of the effective time of the sale event, and all awards with conditions and restrictions relating to the attainment of performance goals may become vested and nonforfeitable in connection with a sale event in the compensation committee's discretion or to the extent specified in the relevant award certificate. Upon the effective time of the sale event, all outstanding awards granted under the 2022 Stock Plan will terminate to the extent not assumed, continued or substituted. In the event of such termination, individuals holding options and stock appreciation rights will be permitted to exercise such options and stock appreciation rights (to the extent exercisable) within a specified period of time prior to the sale event. In addition, in connection with the termination of the 2022 Stock Plan upon a sale event, we may make or provide for a payment, in cash or in kind, to participants holding vested and exercisable options and stock appreciation rights equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights and we may make or provide for a payment, in cash or in kind, to participants holding other vested awards.

Our board of directors will be permitted to amend or discontinue the 2022 Stock Plan and our compensation committee will be permitted to amend the exercise price of options and amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose but no such action may adversely affect rights under an award without the holder's consent. Certain amendments to the 2022 Stock Plan will require the approval of our stockholders. The administrator of the 2022 Plan is specifically authorized to exercise its discretion to reduce the exercise price of outstanding stock options and stock appreciation rights or effect the repricing of such awards through cancellation and re-grants without stockholder consent.

No awards will be granted under the 2022 Stock Plan after the date that is 10 years from the date of stockholder approval. No awards under the 2022 Stock Plan have been made prior to the date of this prospectus.

Employee Stock Purchase Plan

In connection with this offering, our board of directors, upon the recommendation of the compensation committee of the board of directors, or the compensation committee, is expected to adopt the 2021 Employee Stock Purchase Plan, or ESPP, which will be subsequently approved by our stockholders. The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423(b) of the Code. The ESPP initially reserves and authorizes the issuance of up to a total of shares of common stock to participating employees. The ESPP provides that the

number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2023 and each January 1 thereafter through January 1, 2032, by the least of (i) % of the outstanding number of shares of our common stock on the immediately preceding December 31, (ii) shares or (iii) such number of shares of common stock as determined by the ESPP administrator. The number of shares reserved under the ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

All employees whose customary employment is for more than hours per week and have completed at least days of employment are eligible to participate in the ESPP. However, any participating employee who would own 5% or more of the total combined voting power or value of all classes of stock after an option were granted under the ESPP would not be eligible to purchase shares under the ESPP.

We may make one or more offerings each year to our employees to purchase shares under the ESPP. Offerings will begin on such dates as determined by the compensation committee and, unless otherwise determined by the compensation committee, will continue for one year periods, referred to as offering periods. The compensation committee may provide for one or more purchase periods in each offering period. Each eligible employee may elect to participate in any offering by submitting an enrollment form at least 15 business days before the relevant offering date.

Each employee who is a participant in the ESPP may purchase shares of our common stock by authorizing payroll deductions of up to fifteen percent (15%) of his or her base compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions will be used to purchase shares of our common stock on the last business day of the purchase period at a price equal to 85% of the fair market value of the shares of our common stock on the first business day or the last business day of the purchase period, whichever is lower. Under applicable tax rules, an employee may purchase no more than $25,000 worth of shares of common stock, valued at the start of the offering period, under the ESPP in any calendar year.

The accumulated payroll deductions of any employee who is not a participant on the last day of an offering period will be refunded. An employee's rights under the ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment with us for any reason.

Senior Executive Cash Incentive Bonus Plan

In 2022 our board of directors adopted the Senior Executive Cash Incentive Bonus Plan, or the Bonus Plan. The Bonus Plan provides for cash bonus payments based upon the attainment of performance targets established by our compensation committee. The payment targets will be related to financial and operational measures or objectives with respect to our company, or corporate performance goals, as well as individual performance objectives.

Our compensation committee may select corporate performance goals from among the following: developmental, publication, clinical or regulatory milestones; cash flow (including, but not limited to, operating cash flow and free cash flow); revenue; corporate revenue; earnings before interest, taxes, depreciation and amortization; net income (loss) (either before or after interest, taxes, depreciation and/or amortization); changes in the market price of our common stock; economic value-added; acquisitions, licenses or strategic transactions; financing or other capital raising transactions; operating income (loss); return on capital, assets, equity, or investment; stockholder returns; return on sales; total shareholder return; gross or net profit levels; productivity; expense efficiency; margins; operating efficiency; customer satisfaction; working capital; earnings (loss) per share of our common stock; bookings, new bookings or renewals; sales or market shares; number of prescriptions or prescribing physicians; coverage decisions; leadership development, employee retention, and recruiting and other

human resources matters; operating income and/or net annual recurring revenue, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, or as compared to results of a peer group.

Each executive officer who is selected to participate in the Bonus Plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the compensation committee and communicated to each executive. The corporate performance goals will be measured at the end of each performance period after our financial reports have been published or such other appropriate time as the compensation committee determines. If the corporate performance goals and individual performance objectives are met, payments will be made as soon as practicable following the end of each performance period, but not later than 74 days after the end of the fiscal year in which such performance period ends. Subject to the rights contained in any agreement between the executive officer and us, an executive officer must be employed by us on the bonus payment date to be eligible to receive a bonus payment. The Bonus Plan also permits the compensation committee to approve additional bonuses to executive officers in its sole discretion.

401(k) Plan

We maintain a tax-qualified retirement plan that provides eligible U.S. employees with an opportunity to save for retirement on a tax-advantaged basis. Plan participants are able to defer eligible compensation subject to applicable annual Internal Revenue Code limits. We provide a safe-harbor contribution of 3% of employee compensation to employees who satisfy the minimum service requirements. The 401(k) plan is intended to be qualified under Section 401(a) of the Internal Revenue Code with the 401(k) plan's related trust intended to be tax exempt under Section 501(a) of the Internal Revenue Code. As a tax-qualified retirement plan, contributions to the 401(k) plan and earnings on those contributions are not taxable to the employees until distributed from the 401(k) plan.

Nonqualified Deferred Compensation

Our named executive officers did not participate in, or earn any benefits under, a nonqualified deferred compensation plan sponsored by us during fiscal 2020.

Other Benefits

Our named executive officers are eligible to participate in our employee benefit plans on the same basis as our other employees, including our health and welfare plans.

DIRECTOR COMPENSATION

Non-Employee Director Compensation

We did not pay any compensation make any equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2020 for their services as members of the board of directors. As of December 31, 2020, each of Stephen D. Chubb, Walter Gilbert, Ph.D., who resigned from our board of directors in April 2021, and George Mclean Milne Jr., Ph.D. held 2,792, 2,792, and 2,792, respectively of unvested shares of our common stock and held vested options for the following aggregate number of shares of our common stock: 264,950, 125,716, and 106,147, respectively. As of December 31, 2020, each of Felix von Coerper, Dr. Isaac Cheng and Daphne Quimi held no unvested shares of our common stock or options to purchase shares of our common stock. Joshua B. Cohen and Justin Klee received no additional compensation for their service as directors. See the section titled "Executive Compensation" for more information on the compensation paid to or earned by Messrs. Cohen and Klee as employees for the year ended December 31, 2020.

Non-Employee Director Compensation Policy

In connection with this offering, we intend to adopt a new non-employee director compensation policy that will become effective upon the date immediately preceding the date on which the registration statement of which this prospectus is a part is declared effective. The policy will be designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under the policy, each director who is not an employee will be paid cash compensation from and after the completion of this offering as set forth below:

Position	Annual Retainer
Board of Directors:	
Members	$40,000
Audit Committee:	
Members (other than chair)	$ 7,500
Retainer for chair	$15,000
Compensation Committee:	
Members (other than chair)	$ 5,000
Retainer for chair	$10,000
Nominating and Corporate Governance Committee:	
Members (other than chair)	$ 4,000
Retainer for chair	$ 8,000

In addition, the non-employee director compensation policy will provide that, upon initial election to our board of directors, each non-employee director will be granted an equity award of stock options, to purchase shares (the "Initial Grant"). The Initial Grant will vest in one-third on the first anniversary of the date of grant, and the remaining two-thirds will vest in equal monthly installments over two years, provided, however, that all vesting shall cease if the director resigns from the board of directors or otherwise ceases to service as our director. Furthermore, on the date of each of our annual meeting of stockholders upon the completion of this offering, each non-employee director who continues as a non-employee director following such meeting will be granted an annual equity award of stock options, to purchase shares (the "Annual Grant"). The Annual Grant will vest in full upon the earlier of (i) the first anniversary of the date of grant or (ii) the date of the next annual meeting; provided, however, that all vesting shall cease if the director resigns from the board of directors or otherwise ceases to serve as a director, unless the board of directors determines that the circumstances warrant continuation of vesting. In addition, all vested options remain exercisable for twelve (12) months if the director resigns from the board of directors or otherwise ceases to serve as a director. Notwithstanding the foregoing, if an outside director was initially elected to the board of directors within twelve (12) months preceding the annual meeting, then such outside director shall receive an Annual Grant that is pro-rated on a monthly basis for time serving as an outside director. Such Annual Grant shall expire ten years from the date of grant, and shall have a per share exercise price equal to the Fair Market Value (as defined in the 2022 Stock Plan) of the Company's common stock on the date of grant.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

Other than the compensation agreements and other arrangements described under “Executive Compensation” and “Director Compensation” in this prospectus and the transactions described below, since January 1, 2018, there has not been and there is not currently proposed, any transaction or series of similar transactions to which we were, or will be, a party in which the amount involved exceeded, or will exceed, $120,000 and in which any director, executive officer, holder of five percent or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of the foregoing persons, had, or will have, a direct or indirect material interest.

We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that we would pay or receive, as applicable, in arm’s-length transactions.

2017 Convertible Promissory Note Financing

In July 2017, we issued and sold convertible promissory notes to various investors, or the 2017 Notes in the aggregate principal amount of approximately $2.3 million.

The table below sets forth the principal amount of 2017 Notes purchased by related parties since January 1, 2018. In connection with the sale of our Series B preferred stock in June 2020, all such outstanding notes converted into Series B preferred stock in accordance with their terms.

Name	Cash Purchase Price	Number of Shares of Series B Preferred Stock
ALS Invest 1 B.V. (1)	$410,000.00	468,803
Total	$410,000.00	468,803

(1) ALS Invest 1 B.V. holds more than 5% of our voting securities. Felix von Coerper, a member of our board of directors, is a Managing Partner at ALS Investment Fund, an investment fund that is affiliated with ALS Invest 1 B.V.

2018 Convertible Promissory Note Financing

In November 2018, we issued and sold convertible promissory notes to various investors, or the 2018 Notes in the aggregate principal amount of approximately $13.0 million.

The table below sets forth the principal amount of 2018 Notes purchased by related parties. In connection with the sale of our Series B preferred stock in June 2020, all such outstanding notes converted into Series B preferred stock in accordance with their terms.

Name	Cash Purchase Price	Number of Shares of Series B Preferred Stock
ALS Invest 1 B.V. (1)	$4,963,968.00	3,417,113
Morningside Venture Investments Limited (2)	$4,963,968.00	3,417,113
Stephen D. Chubb (3)	$ 220,000.00	151,444
George Mclean Milne Jr, Ph.D. (4)	$ 220,000.00	151,444
Walter Gilbert (5)	$ 100,000	68,838
Total	$ 10,467,936	7,205,952

(1) ALS Invest 1 B.V. holds more than 5% of our voting securities. Felix von Coerper, a member of our board of directors, is a Managing Partner at ALS Investment Fund, an investment fund that is affiliated with ALS Invest 1 B.V.
(2) Morningside Venture Investments Limited holds more than 5% of our voting securities. Isaac Cheng, M.D., a member of our board of directors, is an investment professional at the Morningside Technology Advisory, LLC, a company that is affiliated with Morningside Venter Investments Limited.
(3) Stephen D. Chubb was a member of our board of directors until his resignation in April 2021.
(4) George Mclean Milne Jr, Ph.D. is a member of our board of directors.
(5) Walter Gilbert was a member of our board of directors until his resignation in April 2021.

2020 Convertible Promissory Note Financing

From January 2020 to April 2020, we issued and sold convertible promissory notes to various investors, or the 2020 Notes, in the aggregate principal amount of approximately $15.4 million.

The table below sets forth the principal amount of 2020 Notes purchased by related parties. In connection with the sale of our Series B preferred stock in June 2020, all such outstanding notes converted into Series B preferred stock in accordance with their terms.

Name	Cash Purchase Price	Number of Shares of Series B Preferred Stock
Morningside Venture Investments Limited (1)	$3,644,025.38	240,577
Stephen D. Chubb (2)	$ 250,000.00	16,472
George Mclean Milne Jr, Ph.D. (3)	$ 650,000.00	42,828
Walter Gilbert (4)	$ 250,000.00	16,515
Total	$4,794,025.38	316,392

(1) Morningside Venture Investments Limited holds more than 5% of our voting securities. Isaac Cheng, M.D., a member of our board of directors, is an investment professional at the Morningside Technology Advisory, LLC, a company that is affiliated with Morningside Venter Investments Limited.
(2) Stephen D. Chubb was a member of our board of directors until his resignation in April 2021.
(3) George Mclean Milne Jr, Ph.D. is a member of our board of directors.
(4) Walter Gilbert was a member of our board of directors until his resignation in April 2021.

Series B Preferred Stock Financing

In June 2020, we issued and sold an aggregate of 14,496,835 shares of Series B preferred stock at a price per share of $16.974077, for an aggregate purchase price of approximately $64.4 million. Included in this amount was approximately $34.4 million of outstanding principal and interest on convertible promissory notes issued between July 2017 and April 2020, including the 2017 Notes, 2018 Notes, 2019 Notes and 2020 Notes, all of which converted into Series B preferred stock in this financing in accordance with their terms.

The following table sets forth the aggregate cash purchase price of the Series B preferred stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the number of shares of our Series B preferred stock issued in consideration of such amounts.

Name	Cash Purchase Price	Number of Shares of Series B Preferred Stock
Morningside Venture Investments Limited (1)	$ 26,536,127.62	1,563,333
George Mclean Milne Jr, Ph.D. (2)	$ 200,000.00	11,783
Total	$ 26,736,127.62	1,575,116

(1) Morningside Venture Investments Limited holds more than 5% of our voting securities. Isaac Cheng, M.D., a member of our board of directors, is an investment professional at the Morningside Technology Advisory, LLC, a company that is affiliated with Morningside Venture Investments Limited.
(2) George Mclean Milne Jr, Ph.D. is a member of our board of directors.

The following table sets forth the aggregate principal and interest under the 2017 Notes, 2018 Notes, and 2020 Notes converted by ALS Invest 1 B.V., Morningside Venture Investments Limited, Stephen D. Chubb, George Mclean Milne Jr, Ph.D. and Walter Gilbert, respectively, as our 5% stockholder, director, or executive officer, and the number of shares of our Series B preferred stock issued upon conversion of such securities.

Name	Principal and Interest	Number of Shares of Series B Preferred Stock Received Upon Conversion
ALS Invest 1 B.V. (1)	$ 6,203,147.05	4,178,231
Morningside Venture Investments Limited (2)	$ 9,446,627.79	3,986,541
Stephen D. Chubb (3)	$ 610,181.92	285,369
George Mclean Milne Jr, Ph.D. (4)	$ 1,022,105.02	321,029
Walter Gilbert (5)	$ 449,919.18	173,443
Total	$ 17,731,980.96	8,944,613

(1) ALS Invest 1 B.V. holds more than 5% of our voting securities. Felix von Coerper, a member of our board of directors, is a Managing Partner at ALS Investment Fund, an investment fund that is affiliated with ALS Invest 1 B.V.
(2) Morningside Venture Investments Limited holds more than 5% of our voting securities. Isaac Cheng, M.D., a member of our board of directors, is an investment professional at the Morningside Technology Advisory, LLC, a company that is affiliated with Morningside Venture Investments Limited.
(3) Stephen D. Chubb was a member of our board of directors until his resignation in April 2021.
(4) George Mclean Milne Jr, Ph.D. is a member of our board of directors.
(5) Walter Gilbert was a member of our board of directors until his resignation in April 2021.

2021 Convertible Promissory Note Financing

In January 2021, we issued and sold convertible promissory notes to various investors, or the 2021 Notes, in the aggregate principal amount of approximately $27.3 million.

The table below sets forth the principal amount of 2021 Notes purchased by related parties. In connection with the sale of our Series C preferred stock in July 2021, all such outstanding notes converted into Series C-2 preferred stock in accordance with their terms.

Name	Cash Purchase Price	Number of Shares of Series C-2 Preferred Stock
Morningside Venture Investments Limited (1)	$ 13,972,064.82	1,621,544
George Mclean Milne Jr, Ph.D. (2)	$ 200,000.00	23,212
Walter Gilbert (3)	$ 100,000.00	11,606
Total	$ 14,272,064.82	1,656,362

(1) Morningside Venture Investments Limited holds more than 5% of our voting securities. Isaac Cheng, M.D., a member of our board of directors, is an investment professional at the Morningside Technology Advisory, LLC, a company that is affiliated with Morningside Venture Investments Limited.
(2) George Mclean Milne Jr, Ph.D. is a member of our board of directors.
(3) Walter Gilbert was a member of our board of directors until his resignation in April 2021.

Series C Preferred Stock Financing

In July 2021, we issued and sold an aggregate of 13,150,430 shares of Series C-1 preferred stock at a price per share of $10.265809 and 3,170,585 shares of Series C-2 preferred stock at a price per share of $8.725938, for an aggregate purchase price of approximately $162.7 million. Included in this amount was approximately $27.7 million of outstanding principal and interest on the 2021 Notes,all of which converted into Series C-2 preferred stock in this financing in accordance with their terms.

The following table sets forth the aggregate cash purchase price of the Series C-1 preferred stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the number of shares of our Series C-1 preferred stock issued in consideration of such amounts.

Name	Cash Purchase Price	Number of Shares of Series C-1 Preferred Stock
Morningside Venture Investments Limited (1)	$ 9,999,996.41	974,107
Viking Global Opportunities Illiquid Investments Sub-Master LP (2)	$ 49,999,992.31	4,870,536
George Mclean Milne Jr, Ph.D. (3)	$ 199,998.50	19,482
Justin B. Klee (4)	$ 49,994.49	4,870
Joshua B. Cohen (5)	$ 49,994.49	4,870
Total	$ 60,299,976.20	5,873,865

(1) Morningside Venture Investments Limited holds more than 5% of our voting securities. Isaac Cheng, M.D., a member of our board of directors, is an investment professional at the Morningside Technology Advisory, LLC, a company that is affiliated with Morningside Venture Investments Limited.
(2) Viking Global Opportunities Illiquid Investments Sub-Master LP holds more than 5% of our voting securities and has the contractual right to designate a director to our board of directors.
(3) George Mclean Milne Jr, Ph.D. is a member of our board of directors.
(4) Justin B. Klee is our Co-Chief Executive Officer and a member of our board of directors.
(5) Joshua B. Cohen is our Co-Chief Executive Officer and a member of our board of directors.

The following table sets forth the aggregate principal and interest under the 2021 Notes converted by Morningside Venture Investments Limited, Stephen D. Chubb, George Mclean Milne Jr, Ph.D. and Walter Gilbert, respectively, as our 5% stockholder, director, or executive officer, and the number of shares of our Series C-2 preferred stock issued upon conversion of such securities.

Name	Principal and Interest	Number of Shares of Series C-2 Preferred Stock Received Upon Conversion
Morningside Venture Investments Limited (1)	$14,149,489.76	1,621,544
George Mclean Milne Jr, Ph.D. (2)	$ 202,547.95	23,212
Walter Gilbert (3)	$ 101,273.97	11,606
Total	$14,453,311.68	1,656,362

(1) Morningside Venture Investments Limited holds more than 5% of our voting securities. Isaac Cheng, M.D., a member of our board of directors, is an investment professional at the Morningside Technology Advisory, LLC, a company that is affiliated with Morningside Venture Investments Limited.
(2) George Mclean Milne Jr, Ph.D. is a member of our board of directors.
(3) Walter Gilbert was a member of our board of directors until his resignation in April 2021.

Investors' Rights Agreement

We are a party to an amended and restated investors' rights agreement, dated as of July 1, 2021, or investors' rights agreement, with holders of our preferred stock, including some of our 5% stockholders and entities affiliated with our directors. Such holders consisted of entities affiliated with ALS Invest 1 B.V. Morningside Venture Investments Limited and Viking Global Opportunities Illiquid Investments Sub-Master LP , each a 5% stockholder. Each of ALS Invest 1 B.V. and Morningside Venture Investments Limited has appointed representatives to our board of directors. The investor rights agreement provides these holders the right, following the completion of this offering, to demand that we file a registration statement or request that their shares be covered by a registration statement that we are otherwise filing. See "Description of Capital Stock—Registration Rights" for additional information regarding these registration rights.

Voting Agreement

We are a party to an amended and restated voting agreement, dated as of July 1, 2021, or voting agreement, with holders of our preferred stock, including some of our 5% stockholders and entities affiliated with our directors. Such holders consisted of entities affiliated with ALS Invest 1 B.V. and Morningside Venture Investments Limited, each a 5% stockholder. Each of ALS Invest 1 B.V. and Morningside Venture Investments Limited have appointed representatives to our board of directors. The voting agreement provides the holders the right to elect certain directors to the Board. Pursuant to the voting agreement, we agreed to appoint to our board of directors one representative designated by ALS Invest 1 B.V., Felix von Coerper, one representative designated by Morningside Venture Investments Limited, Isaac S. Cheng, and one representative designated by Viking Global Opportunities Illiquid Investments Sub-Master LP, who will be named at a subsequent date. The voting agreement will terminate upon completion of this offering.

Right of First Refusal and Co-Sale Agreement

We are a party to an amended and restated right of first refusal and co-sale agreement, dated as of July 1, 2021, or right of first refusal and co-sale agreement, with holders of our preferred stock, including some of our 5% stockholders and entities affiliated with our directors. Such holders consisted

of entities affiliated with ALS Invest 1 B.V. Morningside Venture Investments Limited and Viking Global Opportunities Illiquid Investments Sub-Master LP, each a 5% stockholder. Each of ALS Invest 1 B.V. and Morningside Venture Investments Limited have appointed representatives to our board of directors. The right of first refusal and co-sale agreement provides the key holders, as defined in the right of first refusal and co-sale agreement, the right to purchase all or any portion of transfer stock, as defined in the right of first refusal and co-sale agreement, as well as the right of co-sale and to participate in any proposed transfers. The agreement will terminate upon completion of this offering.

Employment Agreements

See the “Executive Compensation—Agreements with Our Named Executive Officers” section of this prospectus for a further discussion of these arrangements.

Indemnification Agreements

Our certificate of incorporation that will become effective as of the closing date of this offering provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we plan to enter into indemnification agreements with each of our officers and directors that may be broader in scope than the specific indemnification provisions contained in the Delaware General Corporation Law. See “Executive Compensation—Limitations on Liability and Indemnification” for additional information regarding these agreements.

Policies and Procedures for Related Person Transactions

Our board of directors reviews and approves transactions with directors, officers and holders of 5% or more of our voting securities and their affiliates, each a related party. Prior to this offering, the material facts as to the related party’s relationship or interest in the transaction are disclosed to our board of directors prior to their consideration of such transaction, and the transaction is not considered approved by our board of directors unless a majority of the directors who are not interested in the transaction approve the transaction. Further, when stockholders are entitled to vote on a transaction with a related party, the material facts of the related party’s relationship or interest in the transaction are disclosed to the stockholders, who must approve the transaction in good faith.

In connection with this offering, we have adopted a written related party transactions policy that such transactions must be approved by our audit committee. This policy will become effective on the date on which the registration statement of which this prospectus is part is declared effective by the SEC. Pursuant to this policy, the audit committee has the primary responsibility for reviewing and approving or disapproving “related party transactions,” which are transactions between us and related persons in which the aggregate amount involved exceeds or may be expected to exceed $120,000 and in which a related person has or will have a direct or indirect material interest. For purposes of this policy, a related person will be defined as a director, executive officer, nominee for director, or greater than 5% beneficial owner of our common stock, in each case since the beginning of the most recently completed year, and their immediate family members. Our audit committee charter will provide that the audit committee shall review and approve or disapprove any related party transactions.

PRINCIPAL STOCKHOLDERS

The following table sets forth information with respect to the beneficial ownership of our common stock as of , 2021 by:

- each of our directors;
- each of our named executive officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock.

The column entitled "Percentage of Shares Beneficially Owned—Before Offering" is based on a total of shares of our common stock outstanding as of , 2021, assuming the automatic conversion of all outstanding shares of our preferred stock into an aggregate of shares of our common stock upon the closing of this offering. The column entitled "Percentage of Shares Beneficially Owned—After Offering" is based on shares of our common stock to be outstanding after this offering, including the shares of our common stock that we are selling in this offering, but not including any additional shares issuable upon exercise of outstanding options or any exercise by the underwriters of their option to purchase additional shares.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock subject to options that are currently exercisable or exercisable within 60 days after , 2021 are considered outstanding and beneficially owned by the person holding the options for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, the persons and entities in this table have sole voting and investment power with respect to all of the shares of our common stock beneficially owned by them, subject to community property laws, where applicable. Except as otherwise set forth below, the address of the beneficial owner is c/o Amylyx Pharmaceuticals, Inc., 43 Thorndike St., Cambridge, MA 02141.

		Percentage of Shares Beneficially Owned	
Name of Beneficial Owner	**Shares Beneficially Owned**	**Before Offering**	**After Offering**
5% Stockholders			
ALS Invest 1 B.V.			
Morningside Venture Investments Limited			
Viking Global Opportunities Illiquid Investments Sub-Master LP			
Named Executive Officers and Directors			
Joshua Cohen			
Justin Klee			
James M. Frates			
Jeffrey Trigilio (1)			
George Mclean Milne Jr, Ph.D.			
Paul Fonteyne			
Margaret Olinger			
Patrick D. Yeramian, M.D.			
Felix Von Coerper			
Isaac Cheng, M.D.			
Daphne Quimi			
All Current Executive Officers and Directors as a Group (ten persons)			

* Represents beneficial ownership of less than 1% of our outstanding stock.
(1) Mr. Trigilio served as our Chief Financial Officer from January 2020 to June 2020.

DESCRIPTION OF CAPITAL STOCK

The following descriptions are summaries of the material terms of our amended and restated certificate of incorporation, which will be effective prior to the closing of this offering and amended and restated bylaws, which will be effective upon the effectiveness of the registration statement of which this prospectus is a part. The descriptions of the common stock and preferred stock give effect to changes to our capital structure that will occur immediately prior to the completion of this offering. We refer in this section to our amended and restated certificate of incorporation as our certificate of incorporation, and we refer to our amended and restated bylaws as our bylaws.

General

Upon completion of this offering, our authorized capital stock will consist of shares of common stock, par value $0.0001 per share, and shares of preferred stock, par value $0.0001 per share, all of which shares of preferred stock will be undesignated.

As of October 31, 2021, 6,799,157 shares of our common stock, 6,289,609 shares of our Series A preferred stock, 14,496,835 shares of our Series B preferred stock, 13,150,430 shares of our Series C-1 preferred stock and 3,170,585 shares of our Series C-2 preferred stock were outstanding and held by 125 stockholders of record.

Common Stock

The holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Preferred Stock

Upon the completion of this offering, all outstanding shares of our preferred stock will be converted into shares of our common stock. Upon the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Registration Rights

Upon the completion of this offering, the holders of 39,474,330 shares of our common stock, including those issuable upon the conversion of preferred stock, will be entitled to rights with respect to the registration of these securities under the Securities Act. These rights are provided under the terms of an amended and restated investors' rights agreement, or the investors' rights agreement, between us and holders of our preferred stock. The investors' rights agreement includes demand registration rights, short-form registration rights and piggyback registration rights. All fees, costs and expenses of underwritten registrations under this agreement will be borne by us and all selling expenses, including underwriting discounts and selling commissions, will be borne by the holders of the shares being registered.

Demand Registration Rights

Beginning 180 days after the effective date of this registration statement, the holders of shares of our common stock, including those issuable upon the conversion of preferred stock, are entitled to demand registration rights. Under the terms of the investors' rights agreement, we will be required, upon the written request of the holders of at least 25% of our outstanding registrable securities, as defined in the investors' rights agreement, or a lesser percent if the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public, net of selling expenses, of least $15.0 million, to file a registration statement and use commercially reasonable efforts to effect the registration of all or a portion of their registrable securities for public resale. We are required to effect only two registrations pursuant to this provision of the investors' rights agreement.

Short-Form Registration Rights

Pursuant to the investor rights agreement, if we are eligible to file a registration statement on Form S-3, upon the written request of the holders of at least 10% of our outstanding registrable securities, as defined in the investors' rights agreement, may demand in writing that we register their registrable securities under the Securities Act so long as the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public, net of selling expenses, of least $3.0 million. We are required to effect only two registrations in any twelve month period pursuant to this provision of the investors' rights agreement. The right to have such shares registered on Form S-3 is further subject to other specified conditions and limitations.

Piggyback Registration Rights

Pursuant to the investors' rights agreement, if we register any of our securities either for our own account or for the account of other security holders, the holders of these shares are entitled to include their shares in the registration. Subject to certain exceptions contained in the investors' rights agreement, we and the underwriters may limit the number of shares included in the underwritten offering to the number of shares which we and the underwriters determine in our sole discretion will not jeopardize the success of the offering.

Indemnification

Our investor rights agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify holders of registrable securities in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions attributable to them.

Expiration of Registration Rights

The demand registration rights and short form registration rights granted under the investor rights agreement will terminate on the fifth anniversary of the completion of this offering.

Anti-Takeover Effects of our Certificate of Incorporation and Bylaws and Delaware Law

Our certificate of incorporation and bylaws include a number of provisions that may have the effect of delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board Composition and Filling Vacancies

Our certificate of incorporation provides for the division of our board of directors into three classes serving staggered three-year terms, with one class being elected each year. Our certificate of incorporation also provides that directors may be removed only for cause and then only by the affirmative vote of the holders of % or more of the shares then entitled to vote at an election of directors. Further, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum. The classification of directors, together with the limitations on removal of directors and treatment of vacancies, has the effect of making it more difficult for stockholders to change the composition of our board of directors.

No Written Consent of Stockholders

Our certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our bylaws or removal of directors by our stockholders without holding a meeting of stockholders.

Meetings of Stockholders

Our certificate of incorporation and bylaws provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our bylaws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Advance Notice Requirements

Our bylaws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days nor more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. Our bylaws specify the requirements as to form and content of all stockholders' notices. These requirements may preclude stockholders from bringing matters before the stockholders at an annual or special meeting.

Amendment to Certificate of Incorporation and Bylaws

Any amendment of our certificate of incorporation must first be approved by a majority of our board of directors, and if required by law or our certificate of incorporation, must thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, board composition, limitation of liability and the amendment of our bylaws and certificate of incorporation must be approved by not less than % of the outstanding shares entitled to vote on the amendment, and not less than % of the outstanding shares of each class entitled to vote thereon as a class. Our bylaws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the bylaws; and may also be amended by the affirmative vote of at least % of the outstanding shares entitled to vote on the amendment, or, if our board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

Undesignated Preferred Stock

Our certificate of incorporation will provide for authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Exclusive Jurisdiction for Certain Actions

Our bylaws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws, or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein. Although we believe this provision benefits us by providing increased consistency in the application of Delaware law in the types of lawsuits to which it applies, the provision may have the effect of discouraging lawsuits against our directors and officers. The enforceability of similar exclusive forum provisions in other companies' bylaws has been challenged in legal proceedings, and it is possible that a court could rule that this provision in our bylaws is inapplicable or unenforceable.

Our bylaws also provide that the United States Federal District Courts will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act or the Exchange Act, unless we consent in writing to an alternative forum, is intended to allow for the consolidation of multi-jurisdiction litigation, avoid state court forum shopping, provide efficiencies in

managing the procedural aspects of securities litigation and reduce the risk that the outcome of cases in multiple jurisdictions could be inconsistent. Although our bylaws contain the choice of forum provisions described above, it is possible that a court could rule that such provisions are inapplicable for a particular claim or action or that such provisions are unenforceable. For example, under the Securities Act, federal courts have concurrent jurisdiction over all suits brought to enforce any duty or liability created by the Securities Act, and investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder.

Section 203 of the Delaware General Corporation Law

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or
- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder; any sale, transfer, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation; subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

Exchange Listing

We have applied to list our common stock on the Nasdaq Global Market under the trading symbol "AMLX."

Transfer Agent and Registrar

The transfer agent and registrar for our common stock will be .

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our shares. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of , upon the completion of this offering shares of our common stock will be outstanding, assuming the automatic conversion of all outstanding shares of our preferred stock into an aggregate of shares of our common stock upon the closing of this offering, no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options or warrants. Of the outstanding shares, all of the shares sold in this offering will be freely tradable, except that any shares held by our affiliates, as that term is defined in Rule 144 under the Securities Act, may only be sold in compliance with the limitations described below. All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, summarized below.

Rule 144

In general, under Rule 144, beginning 90 days after the date of this prospectus, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell shares without restriction, subject to the availability of current public information about us. In addition, under Rule 144, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the date of this prospectus, a person who is our affiliate or who was our affiliate at any time during the preceding three months may sell any unrestricted securities, as well as restricted securities that the person has beneficially owned for at least six months, including the holding period of any prior owner other than one of our affiliates, under Rule 144. Affiliates selling restricted or unrestricted securities may sell a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares then outstanding, which will equal approximately shares immediately after this offering, assuming no exercise of the underwriters' option to purchase additional shares; or
- the average weekly trading volume of our common stock on the Nasdaq Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

Sales under Rule 144 by our affiliates are also subject to manner of sale provisions and notice requirements and to the availability of current public information about us.

Rule 701

In general, under Rule 701 of the Securities Act, any of our employees, consultants or advisors, other than our affiliates, who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement is eligible to resell these shares 90 days after the date of this prospectus in reliance on Rule 144, but without compliance with the holding period requirements of Rule 144 and without regard to the volume of such sales or the availability of public information about us.

However, substantially all Rule 701 shares are subject to lock-up agreements as described below and under “Underwriting” included elsewhere in this prospectus and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Lock-Up Agreements

All of our directors and executive officers and substantially all of our stockholders have signed a lock-up agreement which prevents them from selling any of our common stock or any securities convertible into or exercisable or exchangeable for common stock for a period of not less than 180 days from the date of this prospectus without the prior written consent of Goldman Sachs & Co. LLC, SVB Leerink LLC and Evercore Group L.L.C., subject to certain exceptions. See the section entitled “Underwriting” appearing elsewhere in this prospectus for more information.

Registration Rights

Upon completion of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See the section entitled “Description of Capital Stock—Registration Rights” appearing elsewhere in this prospectus for more information.

Equity Incentive Plans

We intend to file one or more registration statements on Form S-8 under the Securities Act to register our shares issued or reserved for issuance under our equity incentive plans. The first such registration statement is expected to be filed soon after the date of this prospectus and will automatically become effective upon filing with the SEC. Accordingly, shares registered under such registration statement will be available for sale in the open market, unless such shares are subject to vesting restrictions with us or the lock-up restrictions described above.

MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK

The following discussion is a summary of certain U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering. For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes:

- a non-resident alien individual;
- a corporation or any other organization taxable as a corporation for U.S. federal income tax purposes that is created or organized in or under laws other than the laws of the United States, any state thereof, or the District of Columbia;
- an estate, the income of which is not subject to U.S. federal income tax on a net income basis; or
- a trust that (1) has not made an election to be treated as a U.S. person under applicable U.S. Treasury Regulations and (2) either (i) is not subject to the primary supervision of a court within the United States or (ii) is not subject to the substantial control of one or more U.S. persons.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons that hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its tax advisor regarding the tax consequences of holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, or the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any such change or differing interpretation could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset within the meaning of Section 1221 of the Code, which is generally property held for investment.

This discussion does not address all aspects of U.S. federal income taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances including the alternative minimum tax, or the Medicare tax on net investment income, the timing of income accruals required under Section 451(b) of the Code, the rules regarding qualified small business stock within the meaning of Section 1202 of the Code and any election to apply Section 1400Z-2 of the Code to gains recognized with respect to shares of our common stock. This discussion also does not address any U.S. state, local or non-U.S. taxes or any other aspect of any U.S. federal tax other than the income tax. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt or governmental organizations;
- financial institutions;

- brokers or dealers in securities;
- regulated investment companies;
- pension plans;
- “controlled foreign corporations,” “passive foreign investment companies,” and corporations that accumulate earnings to avoid U.S. federal income tax;
- “qualified foreign pension funds,” or entities wholly owned by a “qualified foreign pension fund”;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment;
- persons who have elected to mark securities to market;
- persons who have a functional currency other than the U.S. dollar;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation; and
- certain U.S. expatriates.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock.

Distributions on Our Common Stock

Distributions, if any, on our common stock will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder’s investment, up to such holder’s tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in “Gain on sale or other taxable disposition of our common stock.” Any such distributions will also be subject to the discussions below under the sections titled “Backup withholding and information reporting” and “Withholding and information reporting requirements—FATCA.”

Subject to the discussion in the following two paragraphs in this section, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder’s country of residence. If we or another withholding agent apply over-withholding or if a non-U.S. holder does not timely provide us with the required certification, the non-U.S. holder may be entitled to a refund or credit of any excess tax withheld by timely filing an appropriate claim with the IRS.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a

non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) to the applicable withholding agent and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

Gain on Sale or Other Taxable Disposition of Our Common Stock

Subject to the discussions below under "Backup withholding and information reporting" and "Withholding and information reporting requirements—FATCA," a non-U.S. holder generally will not be subject to any U.S. federal income tax on any gain realized upon such holder's sale or other taxable disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed-base maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed on a net income basis at the U.S. federal income tax rates applicable to United States persons (as defined in the Code) and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above in "Distributions on our common stock" also may apply;
- the non-U.S. holder is a nonresident alien individual who is present in the United States for a period or periods aggregating 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or
- we are, or have been, at any time during the five-year period preceding such sale or other taxable disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder holds no more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rules described above

Backup Withholding and Information Reporting

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such

distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a United States person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above in "Distributions on our common stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them. Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement. Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is filed with the IRS in a timely manner.

Withholding and Information Reporting Requirements—FATCA

Provisions of the Code commonly referred to as the Foreign Account Tax Compliance Act, or FATCA, generally impose a U.S. federal withholding tax at a rate of 30% on payments of dividends on our common stock paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," such foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," such foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Under applicable U.S. Treasury regulations, withholding under FATCA currently applies to payments of dividends on our common stock. Although the FATCA provisions of the Code would require FATCA withholding on gross proceeds, currently proposed U.S. Treasury Regulations provide that FATCA withholding does not apply to gross proceeds from the disposition of property of a type that can produce U.S. source dividends or interest. Taxpayers (including withholding agents) can generally rely on the proposed Treasury Regulations until final Treasury Regulations are issued. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of this withholding tax. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their tax advisors regarding the possible implications of this legislation on their investment in our common stock and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of the 30% withholding tax under FATCA.

UNDERWRITING

We and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman Sachs & Co. LLC, SVB Leerink LLC and Evercore Group L.L.C. are acting as joint book-running managers of this offering and as representatives of the underwriters.

Underwriters	Number of Shares
Goldman Sachs & Co. LLC	
SVB Leerink LLC	
Evercore Group L.L.C.	
H.C. Wainwright & Co., LLC	
Total	

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised.

The underwriters have an option to buy up to an additional shares from us to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by us. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	No Exercise	Full Exercise
Per Share	$	$
Total	$	$

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to $ per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

We and our officers, directors, and holders of substantially all of our common stock have agreed with the underwriters, subject to certain exceptions, not to dispose of or hedge any of their common stock or securities convertible into or exchangeable for shares of common stock during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior written consent of the representatives. See "Shares Available for Future Sale" for a discussion of certain transfer restrictions.

Prior to the offering, there has been no public market for the shares. The initial public offering price has been negotiated among us and the representatives. Among the factors to be considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, will be our historical performance, estimates of the business potential and our earnings prospects, an assessment of our management and the consideration of the above factors in relation to market valuation of companies in related businesses.

An application has been made to quote the common stock on the Nasdaq Global Market under the symbol “AMLX.”

In connection with the offering, the underwriters may purchase and sell shares of common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A “covered short position” is a short position that is not greater than the amount of additional shares for which the underwriters’ option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. “Naked” short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of our stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on the Nasdaq Global Market, in the over-the-counter market or otherwise.

We estimate that our share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately $ million. We have agreed to reimburse the underwriters for certain of their expenses in an amount up to $.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and

other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities or instruments of the issuer (directly, as collateral securing other obligations or otherwise) or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Selling Restrictions

European Economic Area

In relation to each Member State of the European Economic Area, each being a Relevant State, no shares have been offered or will be offered pursuant to the offering to the public in that Relevant State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant State or, where appropriate, approved in another Relevant State and notified to the competent authority in that Relevant State, all in accordance with the Prospectus Regulation, except that the shares may be offered to the public in that Relevant State at any time:

(a) to any legal entity which is a qualified investor as defined under Article 2 of the Prospectus Regulation;

(b) to fewer than 150 natural or legal persons (other than qualified investors as defined under Article 2 of the Prospectus Regulation), subject to obtaining the prior consent of representatives of any such offer; or

(c) in any other circumstances falling within Article 1(4) of the Prospectus Regulation.

provided that no such offer of the shares shall require us or any of the representatives to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation.

For the purposes of this provision, the expression an “offer to the public” in relation to the shares in any Relevant State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares, and the expression “Prospectus Regulation” means Regulation (EU) 2017/1129.

United Kingdom

No shares have been offered or will be offered pursuant to the offering to the public in the United Kingdom prior to the publication of a prospectus in relation to the shares which has been approved by

the Financial Conduct Authority, except that the shares may be offered to the public in the United Kingdom at any time:

(a) to any legal entity which is a qualified investor as defined under Article 2 of the UK Prospectus Regulation;

(b) to fewer than 150 natural or legal persons (other than qualified investors as defined under Article 2 of the UK Prospectus Regulation), subject to obtaining the prior consent of the representatives for any such offer; or

(c) in any other circumstances falling within Section 86 of the Financial Services and Markets Act 2000, or FSMA.

provided that no such offer of the shares shall require the Issuer or any Manager to publish a prospectus pursuant to Section 85 of the FSMA or supplement a prospectus pursuant to Article 23 of the UK Prospectus Regulation. For the purposes of this provision, the expression an "offer to the public" in relation to the shares in the United Kingdom means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares and the expression "UK Prospectus Regulation" means Regulation (EU) 2017/1129 as it forms part of domestic law by virtue of the European Union (Withdrawal) Act 2018.

Canada

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Hong Kong

The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong), or the SFO, of Hong Kong and any rules made thereunder; or (b) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong, or the CO or which do not constitute an offer to the public within the meaning of the CO. No advertisement, invitation or document relating to the shares has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong

Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the SFO and any rules made thereunder.

Singapore

Each joint book-running manager has acknowledged that this prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, each joint book-running manager has represented and agreed that it has not offered or sold any shares or caused the shares to be made the subject of an invitation for subscription or purchase and will not offer or sell any shares or cause the shares to be made the subject of an invitation for subscription or purchase, and has not circulated or distributed, nor will it circulate or distribute, this prospectus or any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares, whether directly or indirectly, to any person in Singapore other than:

(a) to an institutional investor (as defined in Section 4A of the Securities and Futures Act (Chapter 289) of Singapore, as modified or amended from time to time, or the SFA, pursuant to Section 274 of the SFA;

to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA; or

otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

(a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor, securities or securities- based derivatives contracts (each term as defined in Section 2(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:

(i) to an institutional investor or to a relevant person, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;

where no consideration is or will be given for the transfer;

where the transfer is by operation of law; as specified in Section 276(7) of the SFA; or

as specified in Regulation 37A of the Securities and Futures (Offers of Investments) (Securities and Securities-based Derivatives Contracts) Regulations 2018.

Singapore SFA Product Classification—In connection with Section 309B of the SFA and the CMP Regulations 2018, unless otherwise specified before an offer of Notes, we have determined, and hereby notify all relevant persons (as defined in Section 309A(1) of the SFA), that the shares are "prescribed capital markets products" (as defined in the CMP Regulations 2018) and Excluded

Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

Japan

The shares have not been and will not be registered pursuant to Article 4, Paragraph 1 of the Financial Instruments and Exchange Act. Accordingly, none of the shares nor any interest therein may be offered or sold, directly or indirectly, in Japan or to, or for the benefit of, any "resident" of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to or for the benefit of a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Act and any other applicable laws, regulations and ministerial guidelines of Japan in effect at the relevant time.

Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document does not constitute a prospectus within the meaning of, and has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, us or the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

Dubai International Financial Centre

This document relates to an Exempt Offer in accordance with the Markets Rules 2012 of the Dubai Financial Services Authority, or DFSA. This document is intended for distribution only to persons of a type specified in the Markets Rules 2012 of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus supplement nor taken steps to verify the information set forth herein and has no responsibility for this document. The securities to which this document relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the securities offered should conduct their own due diligence on the securities. If you do not understand the contents of this document you should consult an authorized financial advisor. In relation to its use in the Dubai International Financial Centre, or DIFC, this document is strictly private and confidential and is being distributed to a limited number of investors and must not be provided to any person other than the original recipient, and may not be reproduced or used for any other purpose. The interests in the securities may not be offered or sold directly or indirectly to the public in the DIFC.

United Arab Emirates

The shares have not been, and are not being, publicly offered, sold, promoted or advertised in the United Arab Emirates (including the DIFC) other than in compliance with the laws of the United Arab Emirates (and the DIFC) governing the issue, offering and sale of securities. Further, this prospectus does not constitute a public offer of securities in the United Arab Emirates (including the DIFC) and is not intended to be a public offer. This prospectus has not been approved by or filed with the Central Bank of the United Arab Emirates, the Securities and Commodities Authority or the DFSA.

Australia

This prospectus:

- does not constitute a disclosure document or a prospectus under Chapter 6D.2 of the Corporations Act 2001, or the Corporations Act;
- has not been, and will not be, lodged with the Australian Securities and Investments Commission, or ASIC, as a disclosure document for the purposes of the Corporations Act and does not purport to include the information required of a disclosure document for the purposes of the Corporations Act; and
- may only be provided in Australia to select investors who are able to demonstrate that they fall within one or more of the categories of investors, available under section 708 of the Corporations Act, or Exempt Investors.

The shares may not be directly or indirectly offered for subscription or purchased or sold, and no invitations to subscribe for or buy the shares may be issued, and no draft or definitive offering memorandum, advertisement or other offering material relating to any shares may be distributed in Australia, except where disclosure to investors is not required under Chapter 6D of the Corporations Act or is otherwise in compliance with all applicable Australian laws and regulations. By submitting an application for the shares, you represent and warrant to us that you are an Exempt Investor.

As any offer of shares under this document will be made without disclosure in Australia under Chapter 6D.2 of the Corporations Act, the offer of those securities for resale in Australia within 12 months may, under section 707 of the Corporations Act, require disclosure to investors under Chapter 6D.2 if none of the exemptions in section 708 applies to that resale. By applying for the shares you undertake to us that you will not, for a period of 12 months from the date of issue of the shares, offer, transfer, assign or otherwise alienate those shares to investors in Australia except in circumstances where disclosure to investors is not required under Chapter 6D.2 of the Corporations Act or where a compliant disclosure document is prepared and lodged with ASIC.

Bermuda

Shares may be offered or sold in Bermuda only in compliance with the provisions of the Investment Business Act of 2003 of Bermuda which regulates the sale of securities in Bermuda. Additionally, non-Bermudian persons (including companies) may not carry on or engage in any trade or business in Bermuda unless such persons are permitted to do so under applicable Bermuda legislation.

Saudi Arabia

This document may not be distributed in the Kingdom of Saudi Arabia except to such persons as are permitted under the Offers of Securities Regulations as issued by the board of the Saudi Arabian Capital Market Authority, or CMA pursuant to resolution number 2-11-2004 dated 4 October 2004 as

amended by resolution number 1-28-2008, as amended. The CMA does not make any representation as to the accuracy or completeness of this document and expressly disclaims any liability whatsoever for any loss arising from, or incurred in reliance upon, any part of this document. Prospective purchasers of the securities offered hereby should conduct their own due diligence on the accuracy of the information relating to the securities. If you do not understand the contents of this document, you should consult an authorized financial adviser.

British Virgin Islands

The shares are not being, and may not be offered to the public or to any person in the British Virgin Islands for purchase or subscription by or on our behalf. The shares may be offered to companies incorporated under the BVI Business Companies Act, 2004 (British Virgin Islands), or BVI Companies, but only where the offer will be made to, and received by, the relevant BVI Company entirely outside of the British Virgin Islands.

China

This prospectus will not be circulated or distributed in the People's Republic of China, or PRC, and the shares will not be offered or sold, and will not be offered or sold to any person for re-offering or resale directly or indirectly to any residents of the PRC except pursuant to any applicable laws and regulations of the PRC. Neither this prospectus nor any advertisement or other offering material may be distributed or published in the PRC, except under circumstances that will result in compliance with applicable laws and regulations.

Korea

The shares have not been and will not be registered under the Financial Investments Services and Capital Markets Act of Korea and the decrees and regulations thereunder, or the FSCMA, and the shares have been and will be offered in Korea as a private placement under the FSCMA. None of the shares may be offered, sold or delivered directly or indirectly, or offered or sold to any person for re-offering or resale, directly or indirectly, in Korea or to any resident of Korea except pursuant to the applicable laws and regulations of Korea, including the FSCMA and the Foreign Exchange Transaction Law of Korea and the decrees and regulations thereunder, or the FETL. The shares have not been listed on any of securities exchanges in the world including, without limitation, the Korea Exchange in Korea. Furthermore, the purchaser of the shares shall comply with all applicable regulatory requirements (including but not limited to requirements under the FETL) in connection with the purchase of the shares. By the purchase of the shares, the relevant holder thereof will be deemed to represent and warrant that if it is in Korea or is a resident of Korea, it purchased the shares pursuant to the applicable laws and regulations of Korea.

Malaysia

No prospectus or other offering material or document in connection with the offer and sale of the shares has been or will be registered with the Securities Commission of Malaysia, or Commission, for the Commission's approval pursuant to the Capital Markets and Services Act 2007. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Malaysia other than (i) a closed end fund approved by the Commission; (ii) a holder of a Capital Markets Services License; (iii) a person who acquires the shares, as principal, if the offer is on terms that the shares may only be acquired at a consideration of not less than RM250,000 (or its equivalent in foreign currencies) for each transaction; (iv) an individual whose total net personal

assets or total net joint assets with his or her spouse exceeds RM3 million (or its equivalent in foreign currencies), excluding the value of the primary residence of the individual; (v) an individual who has a gross annual income exceeding RM300,000 (or its equivalent in foreign currencies) per annum in the preceding twelve months; (vi) an individual who, jointly with his or her spouse, has a gross annual income of RM400,000 (or its equivalent in foreign currencies), per annum in the preceding twelve months; (vii) a corporation with total net assets exceeding RM10 million (or its equivalent in a foreign currencies) based on the last audited accounts; (viii) a partnership with total net assets exceeding RM10 million (or its equivalent in foreign currencies); (ix) a bank licensee or insurance licensee as defined in the Labuan Financial Services and Securities Act 2010; (x) an Islamic bank licensee or takaful licensee as defined in the Labuan Financial Services and Securities Act 2010; and (xi) any other person as may be specified by the Commission; provided that, in the each of the preceding categories (i) to (xi), the distribution of the shares is made by a holder of a Capital Markets Services License who carries on the business of dealing in securities. The distribution in Malaysia of this prospectus is subject to Malaysian laws. This prospectus does not constitute and may not be used for the purpose of public offering or an issue, offer for subscription or purchase, invitation to subscribe for or purchase any securities requiring the registration of a prospectus with the Commission under the Capital Markets and Services Act 2007.

Taiwan

The shares have not been and will not be registered with the Financial Supervisory Commission of Taiwan pursuant to relevant securities laws and regulations and may not be sold, issued or offered within Taiwan through a public offering or in circumstances which constitutes an offer within the meaning of the Securities and Exchange Act of Taiwan that requires a registration or approval of the Financial Supervisory Commission of Taiwan. No person or entity in Taiwan has been authorized to offer, sell, give advice regarding or otherwise intermediate the offering and sale of the shares in Taiwan.

South Africa

Due to restrictions under the securities laws of South Africa, no "*offer to the public*" (as such term is defined in the South African Companies Act, No. 71 of 2008 (as amended or re-enacted), or the South African Companies Act) is being made in connection with the issue of the shares in South Africa. Accordingly, this document does not, nor is it intended to, constitute a "*registered prospectus*" (as that term is defined in the South African Companies Act) prepared and registered under the South African Companies Act and has not been approved by, and/or filed with, the South African Companies and Intellectual Property Commission or any other regulatory authority in South Africa. The shares are not offered, and the offer shall not be transferred, sold, renounced or delivered, in South Africa or to a person with an address in South Africa, unless one or other of the following exemptions stipulated in section 96 (1) applies:

Section 96(1) (a) the offer, transfer, sale, renunciation or delivery is to:

(i) persons whose ordinary business, or part of whose ordinary business, is to deal in securities, as principal or agent;

(ii) the South African Public Investment Corporation;

(iii) persons or entities regulated by the Reserve Bank of South Africa;

(iv) authorized financial service providers under South African law;

(v) financial institutions recognized as such under South African law;

(vi) a wholly owned subsidiary of any person or entity contemplated in (c), (d) or (e), acting as agent in the capacity of an authorized portfolio manager for a pension fund, or as manager

for a collective investment scheme (in each case duly registered as such under South African law); or

(vii) any combination of the person in (i) to (vi); or

Section 96(1) (b) the total contemplated acquisition cost of the securities, for any single addressee acting as principal is equal to or greater than ZAR1,000,000 or such higher amount as may be promulgated by notice in the Government Gazette of South Africa pursuant to section 96(2)(a) of the South African Companies Act.

LEGAL MATTERS

The validity of the shares of our common stock offered hereby is being passed upon for us by Goodwin Procter LLP, Boston, Massachusetts. Certain legal matters relating to this offering will be passed upon for the underwriters by Wilmer Cutler Pickering Hale and Dorr LLP, New York, New York.

EXPERTS

The financial statements as of December 31, 2019 and 2020, and for each of the two years in the period ended December 31, 2020 included in this Prospectus have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report herein (which report expresses an unqualified opinion on the financial statements and includes an explanatory paragraph referring to the Company's ability to continue as a going concern). Such financial statements have been so included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the common stock we are offering by this prospectus. This prospectus does not contain all of the information included in the registration statement. For further information pertaining to us and our common stock, you should refer to the registration statement and to its exhibits. Whenever we make reference in this prospectus to any of our contracts, agreements or other documents, the references are not necessarily complete, and you should refer to the exhibits attached to the registration statement for copies of the actual contract, agreement or other document.

Upon completion of the offering, you may access, free of charge, our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

Upon the completion of the offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at www.sec.gov. We also maintain a website at www.amylyx.com. Information contained on or accessed through our website is not a part of this prospectus and the inclusion of our website address in this prospectus is an inactive textual reference only.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Amylyx Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Amylyx Pharmaceuticals, Inc. (the “Company”) as of December 31, 2019 and 2020, the related consolidated statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders’ deficit, and cash flows, for each of the two years in the period ended December 31, 2020, and the related notes (collectively referred to as the “financial statements”). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2019 and 2020, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2020, in conformity with accounting principles generally accepted in the United States of America.

Going Concern

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has incurred recurring losses from operations that raise substantial doubt about its ability to continue as a going concern. Management’s plans in regard to these matters are also described in Note 1. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on the Company’s financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company’s internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP
Boston, Massachusetts

April 26, 2021
We have served as the Company’s auditor since 2020.

AMYLYX PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(Amounts in thousands, except share and per share amounts)

	December 31, 2019	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 3,065	$ 12,877
Restricted cash	26	—
Prepaid expenses and other current assets	111	762
Total current assets	3,202	13,639
Property and equipment, net	—	151
Restricted cash	—	189
Other assets	18	125
Total assets	$ 3,220	$ 14,104
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Deficit		
Current liabilities:		
Accounts payable	$ 2,195	$ 3,613
Accrued expenses	2,334	3,713
Total current liabilities	4,529	7,326
Deferred rent	4	14
Accrued interest	335	2
Accrued interest—related parties	881	—
Convertible notes, net of unamortized discount	3,882	—
Convertible notes—related parties, net of unamortized discount	11,049	—
Derivative liability	222	—
PPP Loan	—	263
Proceeds received in advance of issuance of 2021 Notes	—	1,162
Total liabilities	20,902	8,767
Commitments and contingencies (Note 15)		
Series A redeemable convertible preferred stock, $0.0001 par value; 6,401,500 and 6,289,609 shares authorized as of December 31, 2019 and 2020, respectively; 6,289,609 shares issued and outstanding as of December 31, 2019 and 2020, respectively; aggregate liquidation preference of $7,730	7,675	7,675
Series B redeemable convertible preferred stock, $0.0001 par value; 0 and 15,100,000 shares authorized as of December 31, 2019 and 2020, respectively; 0 and 14,496,835 shares issued and outstanding as of December 31, 2019 and 2020, respectively; aggregate liquidation preference of $0 and $246,070 as of December 31, 2019 and 2020, respectively	—	64,387
Stockholders' deficit:		
Common stock, $0.0001 par value; 14,000,000 and 35,000,000 shares authorized as of December 31, 2019 and 2020, respectively; 5,994,246 and 6,137,206 shares issued and outstanding as of December 31, 2019 and 2020, respectively	1	1
Additional paid-in capital	276	1,188
Accumulated deficit	(25,634)	(67,914)
Total stockholders' deficit	(25,357)	(66,725)
Total liabilities, redeemable convertible preferred stock and stockholders' deficit	$ 3,220	$ 14,104

The accompanying notes are an integral part of these consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(Amounts in thousands, except share and per share amounts)

	Year Ended December 31,	
	2019	2020
Grant revenue	$ 1,426	$ 650
Operating expenses:		
Research and development	11,899	24,594
General and administrative	3,081	15,061
Total operating expenses	14,980	39,655
Loss from operations	(13,554)	(39,005)
Other income (expense), net:		
Interest income	176	14
Interest expense	(1,276)	(2,288)
Change in fair value of derivative liability	939	(1,270)
Other (expense) income, net	(1)	269
Total other expense, net	(162)	(3,275)
Net loss and comprehensive loss	(13,716)	(42,280)
Net loss per share attributable to common stockholders—basic and diluted	$ (2.33)	$ (6.96)
Weighted-average shares used in computing net loss per share attributable to common stockholders—basic and diluted	5,889,138	6,077,758

The accompanying notes are an integral part of these consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(Amounts in thousands, except share amounts)

	Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance at December 31, 2018	6,289,609	$ 7,675	—	$ —	5,875,180	$ 1	$ 138	$ (11,915)	$ (11,776)
Cumulative-effect adjustment from adoption of ASU 2018-07	—	—	—	—	—	—	3	(3)	—
Issuance of common stock upon exercise of stock options	—	—	—	—	113,938	—	27	—	27
Vesting of restricted stock awards	—	—	—	—	5,128	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	108	—	108
Net loss	—	—	—	—	—	—	—	(13,716)	(13,716)
Balance at December 31, 2019	6,289,609	$ 7,675	—	$ —	5,994,246	$ 1	$ 276	$ (25,634)	$ (25,357)
Issuance of Series B redeemable convertible preferred stock, net of issuance costs of $35	—	—	1,767,401	29,958	—	—	—	—	—
Conversion of convertible notes and accrued interest into									
Series B redeemable convertible preferred stock	—	—	12,729,434	34,429	—	—	—	—	—
Recognition of contingent beneficial conversion feature							621		621
Issuance of common stock upon exercise of stock options	—	—	—	—	142,960	—	48	—	48
Stock-based compensation expense	—	—	—	—	—	—	243	—	243
Net loss	—	—	—	—	—	—	—	(42,280)	(42,280)
Balance at December 31, 2020	6,289,609	$ 7,675	14,496,835	$ 64,387	6,137,206	$ 1	$ 1,188	$ (67,914)	$ (66,725)

The accompanying notes are an integral part of these consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(Amounts in thousands)

	Year Ended December 31,	
	2019	2020
Cash flows used in operating activities:		
Net loss	$(13,716)	$(42,280)
Adjustments to reconcile net loss to net cash used in operating activities:		
Change in fair value of derivative liability	(939)	1,270
Non-cash interest expense	356	1,738
Stock-based compensation expense	108	243
Depreciation expense	—	1
Gain on extinguishment of convertible notes	—	(268)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	19	(651)
Other assets	(18)	(107)
Accounts payable	1,280	1,418
Accrued expenses, other current liabilities and deferred rent	1,303	1,389
Accrued interest and accrued interest—related parties	920	550
Net cash used in operating activities	(10,687)	(36,697)
Cash flows used in investing activities:		
Purchases of property and equipment	—	(151)
Net cash used in investing activities	—	(151)
Cash flows provided by financing activities:		
Proceeds from PPP Loan	—	263
Proceeds from issuance of convertible notes—related parties	—	4,794
Proceeds from issuance of convertible notes, net of issuance costs	641	10,598
Proceeds from issuance of Series B redeemable convertible preferred stock, net of issuance costs	—	29,958
Proceeds from exercise of stock options	27	48
Proceeds received in advance of issuance of 2021 Notes	—	1,162
Net cash provided by financing activities	668	46,823
Net (decrease) increase in cash, cash equivalents and restricted cash	(10,019)	9,975
Cash, cash equivalents and restricted cash, beginning of period	13,110	3,091
Cash, cash equivalents and restricted cash, end of period	$ 3,091	$ 13,066
Supplemental disclosure of cash flow information:		
Adoption of ASU 2018-07	$ 3	$ —
Recognition of initial derivative liability and associated debt discount	$ 222	$ 4,636
Conversion of convertible notes and accrued interest into Series B redeemable convertible preferred stock	$ —	$ 34,697

The accompanying notes are an integral part of these consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. NATURE OF BUSINESS

Amylyx Pharmaceuticals, Inc. ("Amylyx") was incorporated under the laws of the State of Delaware on January 10, 2014. In October 2020, Amylyx created a wholly owned subsidiary, Amylyx Pharmaceuticals Canada, Inc. ("Amylyx Canada", and together with "Amylyx", the "Company"), in Calgary, Canada. As of December 31, 2020, Amylyx Canada did not have operations. The Company is headquartered in Cambridge, Massachusetts. The Company is a clinical stage biotechnology company with a goal to improve the quality and length of life of patients suffering from neurodegenerative disease. The Company is pursuing commercialization of its asset, AMX0035, which it believes is the first drug candidate to show both a functional and survival benefit in a large-scale clinical trial of patients with amyotrophic lateral sclerosis, or ALS. The Company believes AMX0035 has the potential to be a foundational therapy, meaning a that it could be used alone or in conjunction with other therapies to change the treatment paradigm across a broad range neurodegenerative diseases. The Company has designed AMX0035 to target two key pathways of neuron death, specifically endoplasmic reticulum, or ER, stress and mitochondrial dysfunction. The Company is focused on the development of and potential commercialization of AMX0035 for ALS globally. In addition, the Company is developing AMX0035 for other neurodegenerative diseases by leveraging its unique knowledge and relationships in the neurodegenerative space.

Risks and Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, the outcome of clinical trials, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, ability to secure additional capital to fund operations, and risks associated with the COVID-19 global pandemic, including potential delays associated with the Company's ongoing and anticipated trials. COVID-19 may have an adverse impact on the Company's operations, supply chains and distribution systems or those of its contractors, and increase expenses, including as a result of impacts associated with preventive and precautionary measures that are being taken, such as restrictions on travel and border crossings, quarantine polices and social distancing. The Company and its contractors may experience disruptions in supply of items that are essential for its research and development activities, including, for example, raw materials and bulk drug substances that the Company imports from Europe and Canada used in the manufacturing of AMX0035, and any future product candidates. In addition, the spread of COVID-19 has disrupted global healthcare and healthcare regulatory systems which could divert healthcare resources away from, or materially delay, U.S. Food and Drug Administration ("FDA") approval and approval by other health authorities worldwide with respect to AMX0035 and any future product candidates. Furthermore, the Company's clinical trials may be negatively affected by the COVID-19 outbreak. Site initiation, patient enrollment and patient follow-up visits may be delayed, for example, due to prioritization of hospital resources toward the COVID-19 outbreak, travel restrictions, the inability to access sites for initiation and monitoring, and difficulties recruiting or retaining patients in the Company's ongoing and planned clinical trials.

There can be no assurance that the Company will be able to successfully complete the development of, or receive regulatory approval for, any products developed, and if approved, that any products will be commercially viable. Any products resulting from the Company's current research and development efforts will require significant additional research and development, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts will require significant amounts of additional capital, adequate personnel, infrastructure, and extensive compliance reporting capabilities. The Company has not generated any revenues from the sale of any

products to date. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Going Concern

In accordance with Accounting Standards Update ("ASU") 2014-15, *Disclosure of Uncertainties About an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are conditions and events that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the consolidated financial statements are issued.

Since its inception, the Company has devoted substantially all of its efforts to research and development activities, including recruiting management and technical staff, raising capital, producing materials for non-clinical and clinical studies, and building infrastructure to support such activities. Expenses have primarily been for research and development and related general and administrative costs. The Company has generated revenues through five grants from ALS Association, ALS Finding a Cure Foundation, Cure Alzheimer's Fund, Alzheimer's Drug Discovery Foundation and Alzheimer's Association (collectively, the "Grantors"). In addition to money received from its grants, the Company has also financed its operations through the issuance of redeemable convertible preferred stock and convertible notes (see Notes 9 and 6, respectively). In addition, the Company has financed its operation pursuant to the loan under the Paycheck Protection Program, or the PPP, of the Coronavirus Aid, Relief, and Economic Security ("CARES") Act (the "PPP Loan") as administered by the Small Business Administration ("SBA").

The accompanying consolidated financial statements have been prepared on a going-concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The Company has incurred recurring losses and negative cash flows from operations since inception. As of December 31, 2020, the Company had an accumulated deficit of $67.9 million. The Company expects its operating losses and negative operating cash flows to continue into the foreseeable future as it continues to build capabilities and develop AMX0035, and any future product candidates. These conditions raise substantial doubt regarding the Company's ability to continue as a going concern within one year of the issuance date of the consolidated financial statements. The Company's plans to address the capital shortfall include negotiating additional equity financing and alternative sources of financial support and considering cost containment efforts. Because of the uncertainty inherent in these efforts, the Company has concluded that substantial doubt exists with respect to its ability to continue as a going concern for at least the next twelve months from the date these consolidated financial statements are issued.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation and Consolidation—The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and include the accounts of the Company and its wholly owned subsidiary, Amylyx Canada, after elimination of all significant intercompany accounts and transactions. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and ASU of the Financial Accounting Standards Board ("FASB").

Use of Estimates—The preparation of the consolidated financial statements in conformity with GAAP requires the Company's management to make estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the financial statements and the reported amount of expenses during the reporting period. Actual results could differ from those estimates.

Management considers many factors in selecting appropriate financial accounting policies in developing the estimates and assumptions that are used in the preparation of the financial statements. Management must apply significant judgment in this process. Management's estimation process often may yield a range of potentially reasonable estimates and management must select an amount that falls within that range of reasonable estimates. Estimates are used in the following areas, among others: determining the fair value of the Company's common stock; determining the fair value of derivative liabilities; accrued expenses; stock option valuations; valuation allowance for deferred tax assets and research and development expenses.

Grant Revenue—Grant revenue consists of amounts earned from performing contracted research and development services. The grants between the Company and the Grantors generally provide for the Company to meet certain research milestones in order for funds to be provided. The Company accounts for grant received to perform research and development services in accordance with ASC 730-20, *Research and Development Arrangements*, which requires an assessment, at the inception of the grant, of whether the grant is a liability or a contract to perform research and development services for others. If the Company is obligated to repay the grant funds to the Grantor regardless of the outcome of the research and development activities, then the Company is required to estimate and recognize that liability. Alternatively, if the Company is not required to repay, or if it is required to repay the grant funds only if the research and development activities are successful, then the grant agreement is accounted for as a contract to perform research and development services for others, in which case, grant revenue is recognized as the related research and development expenses are incurred. The Company recognizes grant revenue using the output method that is based on total funds spent relative to the total grant received and receivable. The Company obtained funding from the Grantors of $1.4 million and $0.7 million, which was recorded as grant revenue in the Company's consolidated statements of operations and comprehensive loss during the years ended December 31, 2019 and 2020, respectively. Under the terms of the grants, the Company will be required to pay royalties upon occurrence of contingent future events (see Note 15).

Comprehensive Loss—Comprehensive loss includes net loss, as well as other changes in stockholders' deficit that result from transactions and economic events other than those with stockholders. For the years ended December 31, 2019 and 2020, there were no items, other than net loss, that were included in the Company's comprehensive loss.

Cash and Cash Equivalents—The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. Cash equivalents represent funds invested in readily available checking and money market funds.

Restricted Cash—As of December 31, 2019, the Company maintained a restricted cash account with a balance of less than $0.1 million. The restricted cash as of December 31, 2019 was related to the grant money that the Company received. Such funds were expected to be used within twelve months and as such have been classified as a current asset on the consolidated balance sheet as of December 31, 2019. As December 31, 2020, restricted cash of $0.2 million represents collateral provided for a letter of credit issued as a security deposit in connection with the Company's lease of its corporate office. The lease expires in October 2026 at which time the cash will be released from restriction.

Concentrations of Credit Risk—Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash. Periodically, the Company maintains deposits in an accredited financial institution in excess of federally insured limits. The Company deposits its cash in a financial institution that it believes has high credit quality and has not experienced any losses on such accounts and does not believe it is exposed to any unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

Convertible Note—Derivative—The Company reviews the terms of the convertible note issued to determine whether there are features, including redemption and conversion features, which are required to be bifurcated and accounted for separately as derivative financial instruments. In circumstances where the host instrument contains more than one embedded derivative instrument that is required to be bifurcated, the bifurcated derivative instruments are accounted for as a single, compound derivative instrument.

Bifurcated embedded derivatives are initially recorded at fair value and are then revalued at the end of each reporting period and immediately prior to the conversion or the extinguishment of the convertible note. Changes in the fair value are reported in the consolidated statement of operations and comprehensive loss for the year ended December 31, 2020. When the convertible note contains embedded derivative instruments that are to be bifurcated and accounted for as liabilities, the total proceeds received are first allocated to the fair value of all the bifurcated derivative instruments. The remaining proceeds, if any, are then allocated to the host instruments themselves, usually resulting in those host instruments being recorded at a discount from their face value. The discount from the face value of the convertible note, together with the stated interest on the host instrument, is amortized over the life of the host instrument through periodic charges to interest expense. The Company's convertible notes, as further discussed in Note 6, had embedded derivatives that required bifurcation from the host instrument.

Convertible Note—Beneficial Conversion Feature—If the conversion feature is not treated as a derivative, the Company assesses whether it is a beneficial conversion feature ("BCF"). A BCF exists if the conversion price of the convertible note is less than the price of the stock into which it is convertible to on the commitment date. This typically occurs when the conversion price is less than the fair value of the stock on the date the instrument was issued. The value of a BCF is equal to the intrinsic value of the feature, the difference between the effective conversion price and the fair value of the stock into which it is convertible to and is recorded as additional paid-in capital and as a debt discount in the consolidated balance sheets. The Company amortizes the debt discount as non-cash interest expense over the life of the underlying convertible note using the effective interest method. If the convertible note is retired early, the associated debt discount is then recognized immediately as non-cash interest expense in the consolidated statements of operations and comprehensive. If the conversion feature does not qualify for either the derivative treatment or as a BCF, the convertible note is treated as traditional debt.

Fair Value Measurements—Assets and liabilities recorded at fair value on a recurring basis on the consolidated balance sheet are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

- ***Level 1***—Quoted prices in active markets for identical assets or liabilities.
- ***Level 2***—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- ***Level 3***—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The Company's financial instruments consist of cash, cash equivalents, restricted cash, accounts receivable, accounts payable, accrued expenses and convertible notes. These financial instruments are stated at their respective carrying amounts, which approximate fair value due to the short-term nature of these assets and liabilities. The Company's derivative liability is carried at fair value, determined according to Level 3 inputs in the fair value hierarchy described above (see Note 8).

Debt Issuance Costs—Debt issuance costs associated with the Company's convertible notes are recorded as a reduction of the carrying value of the convertible notes on the Company's consolidated balance sheets and are amortized to interest expense over the term of the respective convertible notes using the effective interest method. During June 2020, all convertible notes converted into shares of Series B redeemable convertible preferred stock. The remaining debt issuance costs were either amortized to non-cash interest expense or recorded to Series B redeemable convertible preferred stock depending on the accounting for the conversion of the convertible notes. For the convertible notes that converted under the conversion feature and for which there was no contingent beneficial conversion feature, the remaining debt issuance costs were recorded as a component of the Series B redeemable convertible preferred stock. For the convertible notes that converted under the conversion feature and for which there was a contingent beneficial conversion feature, the remaining debt issuance costs were recorded as a charge to non-cash interest expense. For the convertible notes that converted under the redemption features, the remaining debt issuance costs were extinguished and recorded as other (expense) income, net.

Property and Equipment, net—Property and equipment are stated at cost, net of accumulated depreciation. Depreciation of property and equipment is calculated using the straight-line method over the estimated useful lives of the respective assets. Maintenance and repairs that do not improve or extend the life of the assets are expensed when incurred. Upon sale or retirement of assets, the cost and accumulated depreciation are removed from the consolidated balance sheets and any resulting gain or loss is reflected in the consolidated statements of operations and comprehensive loss in the period realized. The range of useful lives of property and equipment is as follows:

	Estimated Useful Life
Leasehold improvements	Lesser of the estimated life or remaining lease term
Furniture and fixtures	4 years
Construction in progress	Not depreciated

Impairment of Long-Lived Assets—The Company evaluates assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book values of the assets exceed their fair value. The Company has not recognized any impairment losses in the years ended December 31, 2019 and 2020.

Research and Development—Research and development expenses include costs directly attributable to the conduct of research and development activities. Expenditures relating to research and development are expensed in the period incurred. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the

activity has been performed or when the goods have been received rather than when the payment is made. In addition, research and development related salaries and benefits, facility, and overhead costs, supplies and other related costs are included in research and development expense.

Patent-Related Costs—Patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses in the accompanying consolidated statements of operations and comprehensive loss.

Stock-Based Compensation Expense—The Company accounts for stock-based compensation under the provisions of ASC 718-10, *Compensation—Stock Compensation* , which requires all share-based payments to employees, non-employees and directors, including grants of stock options and restricted stock, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values on the date of grant over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. Generally, the Company issues stock option awards with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company classifies stock-based compensation expense in the same manner in which the awards recipient's payroll or service provider's costs are classified.

The fair value of each restricted common stock award is estimated on the date of grant based on the fair value of the Company's common stock on that same date.

The fair value of each option grant is estimated on the date of grant using the Black-Scholes option pricing model, which requires inputs based on certain subjective assumptions, including the expected stock price volatility, the expected term of the award, the risk-free interest rate, and expected dividends. As there is no public market for the Company's common stock, the estimated fair value of common stock was determined by the Company's Board of Directors as of the date of each option grant, with input from management, considering third-party valuations of its common stock as well as the Company's Board of Directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent third-party valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately Held Company Equity Securities Issued as Compensation*. The Company estimates its expected stock price volatility based on the historical volatility of publicly traded peer companies. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. There is no expected dividend yield since the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future.

Contingencies—From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of business activities. The Company accrues for loss contingencies when losses become probable and are reasonably estimable. If the reasonable estimate of the loss is a range and no amount within the range is a better estimate, the minimum amount of the range is recorded as a liability on the Company's consolidated balance sheets. The Company does not accrue for contingent losses that, in its judgement, are considered to be reasonably possible, but not probable; however, it discloses the range of reasonably possible losses. There were no loss or gain contingencies recorded in the Company's consolidated financial statements as of and during the year ended December 2019 and 2020.

Leases—The Company leases its office, and may from time to time, enter into other lease agreements in conducting its business. At the inception of each lease, the Company evaluates the lease agreement to determine whether the lease is an operating or capital lease in accordance with ASC 840, *Leases* (ASC 840). When any one of the four test criteria in ASC 840 is met, the lease then qualifies as a capital lease. If the lease agreements contain renewal options, tenant improvement allowances, rent holidays or rent escalation clauses, the Company records a deferred rent asset or liability equal to the difference between the rent expense and future minimum lease payments due. The rent expense related to operating leases is recognized on a straight-line basis in the statements of operations and comprehensive loss over the term of each lease. The Company did not have capital leases as of December 31, 2019 and 2020.

Income Taxes—The Company accounts for income taxes using the asset and liability approach. Deferred tax assets and liabilities represent future tax consequences of temporary differences between the financial statement carrying amounts and the tax basis of assets and liabilities and for loss carryforwards using enacted tax rates expected to be in effect in the years in which the differences reverse. A valuation allowance is established to reduce deferred tax assets to the amounts expected to be realized. The Company also recognizes a tax benefit from uncertain tax positions only if it is "more likely than not" that the position is sustainable based on its technical merits. The Company accounts for interest and penalties related to uncertain tax positions as part of its provision for income taxes. To date, the Company has not incurred interest and penalties related to uncertain tax positions.

Segment Information—An operating segment is defined as a component of a business that engages in business activities for which it may earn revenues and incur expenses and for which discrete financial information is available that is evaluated regularly by the chief operating decision maker or makers in order to make decisions about resources to be allocated to the segment and assess its performance. The Company operates and manages the business as one reporting and one operating segment, which is the business of developing therapeutics for neurodegenerative disorders. The Company has determined that its chief executive officers are the chief operating decision maker ("CODM"). The CODM reviews consolidated operating results to make decisions about allocating resources or capital to specific compounds or projects in line with the Company's overall strategies and goals. As of December 31, 2019, the Company operated in one geographic region in the United States. As of December 31, 2020, the Company had offices in two geographic regions in United States and Canada, but operations were only in the United States.

Net income (loss) per share—The Company follows the two-class method when computing net income (loss) per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net income (loss) per share attributable to common stockholders is computed by dividing the net income (loss) attributable to common stockholders by the weighted average number of common shares outstanding for the period. Diluted net income (loss) attributable to common stockholders is computed by adjusting net income (loss) attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net income (loss) per share attributable to common stockholders is computed by dividing the diluted net income (loss) attributable to common stockholders by the weighted average number of common shares outstanding for the period, including potential dilutive common shares. For purpose of this calculation, stock options, convertible notes, and redeemable convertible preferred stock are considered potential dilutive common shares.

The Company's redeemable convertible preferred stock contractually entitles the holders of such shares to participate in dividends but does not contractually require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss attributable to common stockholders, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive.

Emerging Growth Company Status—The Company is an emerging growth company ("EGC") as defined in the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act"), and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not EGCs. The Company may take advantage of these exemptions until it is no longer an EGC under Section 107 of the JOBS Act and has elected to use the extended transition period for complying with new or revised accounting standards. As a result of this election, the Company's consolidated financial statements may not be comparable to companies that comply with public company FASB standards' effective dates. The Company intends to take advantage of the reduced reporting requirements and exemptions up until the last day of the fiscal year following the fifth anniversary of an offering or such earlier time that it is no longer an EGC.

Recent Accounting Pronouncements

New Accounting Pronouncements Not Yet Adopted—In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* ("ASC 842"), which sets out the principles for the recognition, measurement, presentation, and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, for finance and operating leases, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. ASC 842 provides a lessee with an option to not account for leases with a term of 12 month or less as leases in the scope of the new standard. ASC 842 supersedes the previous leases standard, ASC 840 *Leases*. In July 2018, the FASB issued supplemental adoption guidance and clarification to ASC 842 within ASU No. 2018-10, *Codification Improvements to Topic 842, Leases* and ASU No. 2018-11, *Leases (Topic 842): Targeted Improvements*. ASU No. 2018-11 provides another transition method in addition to the existing modified retrospective transition method by allowing entities to initially apply the new leasing standard at the adoption date and recognize a cumulative-effect adjustment to the opening balance of accumulated deficit in the period of adoption. In July 2019, the FASB delayed the effective date for this ASU for non-public entities (including emerging growth companies), and this ASU is effective for fiscal years beginning after December 15, 2021 and interim periods within fiscal years beginning after December 15, 2022, with early adoption permitted. The ASU is effective for the Company on January 1, 2022 and the Company intends to adopt the ASU when it becomes effective. The Company is currently evaluating the impact the adoption of these ASUs will have on its consolidated financial statements and related disclosures. The Company expects to recognize a right-of-use asset and corresponding lease liability for its real estate operating leases upon adoption. See Note 15 for more information related to the Company's lease obligations.

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326) Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"), which requires an entity to utilize a new impairment model known as the current expected credit loss model to estimate its lifetime "expected credit loss" and record an allowance that, when deducted from the amortized cost

basis of the financial assets and certain other instruments, including but not limited to available-for-sale debt securities. Credit losses relating to available-for-sale debt securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. ASU 2016-13 requires a cumulative effect adjustment to the consolidated balance sheet as of the beginning of the first reporting period in which the guidance is effective. In November 2019, the FASB issued ASU 2019-10, *Financial Instruments—Credit Losses (Topic 326), Derivatives and Hedging (Topic 815) and Leases (Topic 842): Effective Dates*, which defers the effective date of ASU 2016-13 to fiscal years beginning after December 15, 2022 for all entities except Securities and Exchange Commission filers that are not smaller reporting companies. ASU 2016-13 will be effective for the Company beginning January 1, 2023. The Company intends to adopt the ASU when it becomes effective. The Company is currently evaluating the impact of this ASU and does not expect that adoption of this standard will have a material impact on its consolidated financial statements and related disclosures.

In August 2020, the FASB issued Accounting Standards Update 2020-06, *Accounting for Convertible Instruments and Contracts in an Entity's Own Equity", ("ASU 2020-06")*. ASU 2020-06 simplifies the accounting for certain financial instruments with characteristics of liabilities and equity, including convertible instruments and contracts on an entity's own equity. ASU 2020-06 removes from U.S. GAAP the separation models for (i) convertible debt with a cash conversion feature and (ii) convertible instruments with a beneficial conversion feature. As a result, after adopting the ASU's guidance, entities will not separately present in equity an embedded conversion feature in such debt. Instead, the entity will account for a convertible debt instrument wholly as debt, and for convertible preferred stock wholly as preferred stock (i.e., as a single unit of account), unless (1) a convertible instrument contains features that require bifurcation as a derivative under ASC 815 or (2) a convertible debt instrument was issued at a substantial premium. In addition, the ASU also states that entities must apply the if-converted method to all convertible instruments for calculation of diluted earnings per share and the treasury stock method is no longer available.

ASU 2020-06 is effective for all public entities, excluding smaller reporting entities, for fiscal years beginning after December 15, 2021, including interim periods within those fiscal years. For all other entities, this ASU is effective for fiscal years beginning after December 15, 2023, including interim periods within those fiscal years. Early adoption is permitted for fiscal years beginning after December 31, 2020. The Company expects to early adopt this guidance beginning from January 1, 2021. The Company is currently assessing the impact that this ASU will have on its consolidated financial statements and related disclosures.

Recently Adopted Accounting Pronouncements—In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers (Topic 606)* ("ASU 2014-09"). Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The ASU also states that an entity should recognize as an asset the incremental costs of obtaining a contract that the entity expects to recover and amortize that cost over a period consistent with the period over which the transfer to the customer of the underlying good or services occurs. The Company adopted ASC 606 on January 1, 2019 utilizing the modified retrospective method of transition. Accordingly, the consolidated financial statements for the years ended December 31, 2019 and 2020 are presented under ASC 606. The adoption of the new standard did not result in a material change in the timing or amount of revenue recognized.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement* ("ASU 2018-13"). The ASU modifies, and in certain cases eliminates, the disclosure requirements on fair value measurements in Topic 820. The amendments in ASU No. 2018-13 were effective for the Company on January 1, 2020. Early adoption is permitted. An entity is permitted to early adopt any removed or modified disclosures upon issuance of ASU No. 2018-13 and delay adoption of the additional disclosures until their effective date. The Company early adopted this standard on January 1, 2019 and it had no impact on its consolidated financial statements and footnote disclosures.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation-Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07"). ASU 2018-07 amends the FASB ASC to expand the scope of FASB ASC Topic 718, *Compensation-Stock Compensation*, to include accounting for share-based payment transactions for acquiring goods and services from non-employees. The amendments in ASU 2018-07 are effective for all entities for annual periods, and interim periods within those annual periods, beginning after December 15, 2018. The Company adopted this guidance on January 1, 2019. After the adoption of ASU 2018-07, the measurement date for nonemployee awards is the later of the adoption date of ASU 2018-07 or the date of grant, without recognition for changes in the fair value of the award. Stock-based compensation costs for nonemployees are recognized as expense over the vesting period on a straight-line basis. The adoption of this standard did not have a material impact on the Company's financial position, results of operations or cash flows.

In August 2018, the FASB issued ASU 2018-13, which eliminates certain disclosure requirements for fair value measurements for all entities, requires public entities to disclose certain new information and modifies some disclosure requirements. The Company adopted this standard on January 1, 2020 and it did not have a material impact on its consolidated financial statements.

3. PROPERTY AND EQUIPMENT, NET

Property and equipment, net consisted of the following:

	December 31, 2020 (in thousands)
Furniture and fixtures	$ 49
Leasehold improvements	41
Construction in progress	62
Total property and equipment	152
Less: accumulated depreciation	(1)
Total property and equipment, net	$ 151

The Company did not have any property and equipment as of December 31, 2019.

4. ACCRUED EXPENSES

Accrued expenses consisted of the following:

	December 31, 2019	December 31, 2020
	(in thousands)	
External research and development	$1,421	$ 293
Payroll and employee related expenses	652	1,855
Accrued legal and other professional fees	141	584
Other accrued expenses	120	981
Total accrued expenses	$2,334	$3,713

5. PPP LOAN

In April 2020, the Company obtained a PPP Loan from First Republic Bank in the aggregate amount of $0.3 million, which was established under the CARES Act. Under the terms of the CARES Act and the PPP, all or a portion of the principal amount of the PPP Loan is subject to forgiveness so long as, over the 24-week period following the Company's receipt of the proceeds of the PPP Loan, the Company uses those proceeds for payroll costs, rent, utility costs or the maintenance of employee and compensation levels. The PPP Loan is unsecured, guaranteed by the SBA, and has a two-year term, maturing in April 2022. Interest accrues on the PPP Loan beginning with the initial disbursement. The application for the forgiveness of the PPP Loan can be made during an 8-week period beginning from the date of initial disbursement. Unless forgiven in whole or in part in accordance with the PPP regulation, the terms of the PPP Loan provide for the Company to make monthly payments of the principal and interest on the outstanding principal balance of the PPP Loan equal to the balance of the PPP Loan amortized over the term of the PPP Loan beginning seven (7) months from the initial disbursement until maturity.

The terms of the PPP loan provide for customary events of default including, among other things, payment defaults, breach of representations and warranties, and insolvency events. The Company has determined to account for the PPP Loan as debt under ASC 470, *Debt*. As of December 31, 2020, the outstanding balance of the PPP Loan was $0.3 million.

6. CONVERTIBLE NOTES

Convertible Notes, Net of Unamortized Discount

Convertible notes, net of unamortized discount consisted of the following:

	December 31, 2019
	(in thousands)
Principal value of convertible notes	$ 15,962
Note discount	(1,031)
Convertible notes, net of unamortized discount	$ 14,931

In June 2020, the convertible notes converted into Series B redeemable convertible preferred stock. There were no convertible notes outstanding as of December 31, 2020.

Issuance of the 2017 Notes, 2018 Notes, 2019 Notes and 2020 Notes (collectively, the "Notes")

In July 2017, the Company commenced an offering to issue $2.3 million convertible notes ("2017 Notes") to certain investors with a maturity date of December 31, 2021. These 2017 Notes carried both a voluntary conversion feature and an automatic conversion feature. The 2017 Notes were secured and carried an interest rate of 6%.

In November 2018, the Company commenced an offering to issue $13.0 million convertible notes ("2018 Notes") with a maturity date of December 31, 2021. These 2018 Notes carried both a voluntary conversion feature and an automatic conversion feature. The 2018 Notes were secured and carried an interest rate of 6%.

In December 2019, the Company issued $0.6 million of convertible notes ("2019 Notes") to certain investors with a maturity date of December 31, 2021. These 2019 Notes carried an automatic conversion feature only. The 2019 Notes were secured and carried an interest rate of 2%.

In January, February and April 2020, the Company issued, in aggregate, $15.4 million in convertible notes ("2020 Notes") to certain investors with a maturity date of December 31, 2021. These 2020 Notes carried an automatic conversion feature only. The 2020 Notes were secured and carried an interest rate of 2%.

The Notes contained the following features:

Automatic Conversion Features —The Notes were to automatically convert into Conversion Shares upon (i) the sale of substantially all of the assets of the Company ("Asset Sale"), (ii) the occurrence of a transaction or series of transactions in which holders of 100% of the Company's outstanding shares of stock immediately before such transaction held 50% or less of the outstanding shares of the Company's stock or the surviving corporation immediately after such transaction ("Stock Sale"), and (iii) sale of equity securities of any kind after the issuance of the Notes for which the Company had received consideration of at least $5.0 million ("Financing", and together with the Asset Sale and Stock Sale, collectively, "Triggering Events"). In the event of an Asset Sale or Stock Sale, the Conversion Shares would be Common Stock of the Company. In the event of a Financing event, the Conversion Shares would be the class of equity shares issued in such transaction.

Conversion Price—Upon the occurrence of a Triggering Event, the 2017 Notes would convert based on the amount equal to the lesser of (i) 85% of the share price paid by the investors in the Financing, Asset Sale or Stock Sale and (ii) $25.0 million divided by the fully diluted capital. Upon the occurrence of a Triggering Event, the 2018 Notes, would convert based on the amount equal to the lesser of (i) 85% of the share price paid by the investors in the Financing, Asset Sale or Stock Sale and (ii) $30.0 million divided by the fully diluted capital. Upon the occurrence of a Triggering Event, the 2019 and 2020 Notes would convert based on the amount equal to 90% of the share price paid by the investors in the Financing, Asset Sale or Stock Sale.

Voluntary Conversion Feature—Under the terms of the 2017 Notes, the holders of the 2017 Notes had the option to convert their notes at any time prior to maturity into shares of the Company's common stock at a conversion price equal to the $25.0 million divided by the fully diluted capital, provided their notes had not been previously converted pursuant to a Triggering Event.

Under the terms of the 2018 Notes, the holders of the 2018 Notes had the option to convert their notes at any time prior to the maturity into shares of the Company's common stock at a conversion price equal to the $30.0 million divided by the fully diluted capital, provided their notes had not been previously converted pursuant to a Triggering Event.

Embedded Derivatives

The Company assessed all the terms of the Notes in order to identify any potential embedded features and determined that the following redemption features required bifurcation and separate accounting as derivatives: automatic conversion feature in connection with a Financing, Asset Sale and Stock Sale. For the respective Notes, the Company bundled these features together and accounted for the features as a single, compound embedded derivative. The Company determined the fair value of the embedded derivative as the difference between the estimated fair value of the respective Notes with and without the redemption features, which resulted in the Company recording the respective Notes at a discount.

The fair value of the bifurcated embedded derivatives as of the respective issuance dates of the 2017 Notes, 2018 Notes, 2019 Notes and 2020 Notes was determined to be $0.3 million, $0.9 million, $0.2 million, and $4.6 million, respectively.

The Company amortized the debt discount over the contractual life of the Notes as a non-cash interest expense utilizing the effective interest method.

At each financial reporting period, and immediately prior to conversion, the Company remeasured the fair value of the derivative liability bifurcated from the Notes and recognized changes in the fair value of derivative liability in the statements of operations and comprehensive loss. As of December 31, 2019, the derivative related to the 2017 Notes and 2018 Notes had no fair value as (i) the conversion under Asset Sale and Stock Sale scenario was eliminated because these scenarios were not likely to occur and (ii) there was no difference between the fair value of the 2017 Notes and 2018 Notes with and without the redemption features.

As of December 31, 2019, the fair value of the derivative liability related to the 2019 Notes was $0.2 million. As of December 31, 2020, there was no derivative liability due to the conversion of the Notes in June 2020.

Conversion of the Notes

In June 2020, the Company consummated a financing transaction in which it issued shares of Series B redeemable convertible preferred stock. The consummation of this financing transaction resulted in the automatic conversion of the Notes into shares of Series B redeemable convertible preferred stock pursuant to their original terms. The Company accounted for the conversion of the 2017 Notes and 2018 Notes as a conversion as these notes converted pursuant to the conversion features in their original terms. The 2017 Notes and 2018 Notes converted into 2,737,494 and 8,933,907 shares of Series B redeemable convertible preferred stock, respectively, at the effective conversion prices of $0.9988 and $1.5929, respectively. The 2019 Notes and 2020 Notes converted into 42,366 and 1,015,667 shares of Series B redeemable convertible preferred stock, respectively, at the conversion price of $15.2767.

The 2017 Notes and 2018 Notes contained contingent beneficial conversion features, which were not readily determinable upon the issuance of the notes because the conversion features were contingent upon the occurrence of an undetermined future financing transaction and neither the timing, including the type of security that would be issued in such transaction, nor the value of such transaction could be estimated at the time the notes were issued. Upon the automatic conversion of the 2017 Notes in connection with the consummation of the financing transaction that resulted in the issuance of the Series B redeemable convertible preferred stock, the Company initially recorded $0.6 million as a debt discount that was immediately recognized through interest expense with an offset to additional paid-in capital to reflect the contingent beneficial conversion feature associated with

the conversion of the 2017 Notes into Series B redeemable convertible preferred stock. The intrinsic value of the beneficial conversion feature was calculated as the difference between (i) the effective conversion price for the 2017 Notes, which was represented by the price at which the 2017 Notes converted into Series B redeemable convertible preferred stock and (ii) the fair value of the existing Series A redeemable convertible preferred stock at the original commitment date (which is the only class available to benchmark to). Additionally, as the 2017 Notes included a contingent beneficial conversion feature, the Company recognized all of the unamortized discount remaining at the date of conversion relating to the original allocation of proceeds to the bifurcated derivative to interest expense. This amounted to $0.1 million and resulted in an increase in the carrying value of the 2017 Notes and an immediate charge to non-cash interest expense in the consolidated statements of operations and comprehensive loss. Also, there were no beneficial conversion features recorded for the Series B redeemable convertible preferred stock, which are convertible into common stock at any time, issued upon the conversion of the 2017 Notes as there was no intrinsic value.

Upon the automatic conversion of the 2018 Notes in connection with the consummation of the financing transaction that resulted in the issuance of the Series B redeemable convertible preferred stock, the Company did not record beneficial conversion feature for the 2018 Notes as there was no intrinsic value attributed to the contingent beneficial conversion feature.

The fair value of the Series B redeemable convertible preferred stock issued upon conversion of the 2017 Notes and the proceeds allocated to the 2017 Notes were $46.5 million and $2.6 million, respectively. The fair value of the Series B redeemable convertible preferred stock issued upon conversion of the 2018 Notes and the proceeds allocated to the 2018 Notes were $151.6 million and $13.7 million, respectively. The Series B redeemable convertible preferred stock issued upon conversion of the Notes and in exchange for cash payment did not contain a recognized beneficial conversion feature at the time of issuance, but the shares do contain a contingent beneficial conversion feature to the extent the security's conversion price decreases below the commitment date fair value of the Company's common stock.

Upon conversion of the 2017 Notes and 2018 Notes, the Company derecognized the carrying values of these notes including accrued interest of $16.4 million and recognized Series B redeemable convertible preferred stock.

The Company accounted for the conversion of the 2019 Notes and 2020 Notes as an extinguishment as these notes converted pursuant to redemption features that were bifurcated as embedded derivatives at the original commitment date. The Company recorded a gain on extinguishment of convertible notes of $0.3 million, which is included in other expense, net in the statements of operations and other comprehensive loss. The gain on extinguishment of convertible notes is the excess of (i) the total carrying value of the 2019 Notes and 2020 Notes including accrued interest of $12.2 million and the derivative liability of $6.1 million over (ii) the fair value of the shares of Series B redeemable convertible preferred stock into which the 2019 Notes and 2020 Notes converted of $18.0 million.

Convertible Notes—Related Parties

Convertible notes—related parties, net of unamortized discount consisted of the following:

	December 31, 2019
	(in thousands)
Principal value of convertible notes	$ 11,691
Note discount	(642)
Convertible notes—related parties, net of unamortized discount	$ 11,049

There were no convertible notes issued to related parties that were outstanding as of December 31, 2020.

In connection with the issuance of the 2017 Notes, the Company issued, in aggregate, $1.2 million of convertible notes to certain related parties. These notes were issued under the same terms and conditions as the 2017 Notes. In June 2020, these convertible notes converted into Series B redeemable convertible preferred stock together with the 2017 Notes.

In connection with the issuance of the 2018 Notes, the Company issued, in aggregate, $10.5 million of convertible notes to certain related parties. These notes were issued under the same terms and conditions as the 2018 Notes. In June 2020, these convertible notes converted into Series B redeemable convertible preferred stock together with the 2018 Notes.

In connection with the issuance of the 2020 Notes, the Company issued, in aggregate, $4.8 million of convertible notes to certain related parties. These notes were issued under the same terms and conditions as the 2020 Notes. In June 2020, these convertible notes converted into Series B redeemable convertible preferred stock together with 2020 Notes.

7. PROCEEDS RECEIVED IN ADVANCE OF ISSUANCE OF 2021 NOTES

In January 2021, the Company entered into subscription agreements with certain investors pursuant to which the Company would issue convertible notes (“2021 Notes”) in the aggregate amount of $27.3 million thereafter. In advance of the issuance of the 2021 Notes, the Company received $1.2 million in proceeds from certain investors in December 2020. The Company recorded these proceeds as proceeds received in advance of issuance of 2021 Notes on the consolidated balance sheet as of December 31, 2020, as the subscription agreement and commitment to issue notes was not effective until January 2021 and the amount is not reasonably expected to be repaid by using the Company’s current assets or creating current liabilities.

8. FAIR VALUE MEASUREMENTS

The following table presents information about the Company’s financial assets and liabilities measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values:

	December 31, 2019			
	Level 1	Level 2	Level 3	Total
		(in thousands)		
Assets:				
Money market funds	$ 2,853	$ —	$ —	$ 2,853
Total financial assets	$ 2,853	$ —	$ —	$ 2,853
Liabilities:				
Derivative liability	$ —	$ —	$ 222	$ 222
Total financial liabilities	$ —	$ —	$ 222	$ 222

	December 31, 2020			
	Level 1	Level 2	Level 3	Total
		(in thousands)		
Assets:				
Money market funds	$10,004	$ —	$ —	$10,004
Restricted cash	189	—	—	189
Total financial assets	$10,193	$ —	$ —	$10,193

The fair value of the derivative liabilities was measured using a with-and-without valuation methodology. Inputs used to determine the estimated fair value of the derivative instruments include the probability estimates of potential settlement scenarios for the convertible promissory notes, a present value discount rate and an estimate of the expected timing of settlement. Certain unobservable inputs used in the fair value measurement of the derivative instruments associated with the convertible promissory notes are the scenario probabilities and the discount rate estimated at the valuation date. Generally, an increase or decrease in the discount rate would result in a directionally opposite impact to the fair value measurement of the derivative instruments. Also, changes in the probability scenarios would have varying impacts depending on the weighting of each specific scenario. Heavier weighting toward a qualified financing would result in an increase in the fair value of the derivative liability. Changes in these assumptions can materially affect the fair value.

An initial fair value valuation was performed at each date of issuance of the outstanding convertible notes and subsequently remeasured as of each reporting period and immediately prior to conversion. The change in fair value between measurement dates was determined to be a gain of $0.9 million and a loss of $1.3 million for the years ended December 31, 2019 and 2020, respectively, which was recognized in the consolidated statements of operations and comprehensive loss.

The following table set forth the significant inputs to the probability weighted valuation model used to value the derivative liability as of December 31, 2019:

Type of Event	Expected Date	Probability of Event	Discount Rate	Convertible Notes
Qualified Financing	March 31, 2020	47.8%	1.59%	2017 Notes and 2018 Notes
Stock or Asset Sale	December 31, 2022	52.2%	0%	2017 Notes and 2018 Notes
Note Reaches Maturity	December 31, 2021	0%	30%	2017 Notes and 2018 Notes
Qualified Financing				2019 Notes
	June 30, 2020	75.0%	30%	
Stock or Asset Sale				2019 Notes
	March 31, 2021	5.0%	30%	
Note Reaches Maturity				2019 Notes
	December 31, 2021	20.0%	30%	

The following table set forth the significant inputs to the probability weighted valuation model used to value the derivative liability at issuance of the 2020 Notes:

Type of Event	Expected Date	Probability of Event	Discount Rates	Convertible Notes
Qualified Financing	June 30, 2020	75.0%	30%	2020 Notes
Stock or Asset Sale	March 31, 2021	5.0%	30%	2020 Notes
Note Reaches Maturity	December 31, 2021	20.0%	30%	2020 Notes

The following table set forth the significant inputs to the probability weighted valuation model used to value the derivative liability upon conversion of the Notes in June 2020:

Type of Event	Expected Date	Probability of Event	Discount Rates	Convertible Notes
Qualified Financing	June 18, 2020	100%	25%	2017, 2018, 2019 and 2020 Notes
Stock or Asset Sale	n/a	0%	25%	2017, 2018, 2019 and 2020 Notes
Note Reaches Maturity	n/a	0%	25%	2017, 2018, 2019 and 2020 Notes

There were no other assets or liabilities that were measured at fair value on a recurring basis as of December 31, 2020.

The following table presents changes in the derivative liabilities with significant unobservable inputs (Level 3):

	Derivative Liability (in thousands)
Balance as of January 1, 2019	$ 939
Decrease in derivative liability resulting from change in estimated fair value	(939)
Increase in derivative liability resulting from issuance of convertible notes	222
Balance as of December 31, 2019	$ 222
Increase in derivative liability resulting from issuance of convertible notes	4,636
Increase in derivative liability resulting from change in estimated fair value	1,270
Derivative liability settled upon conversion of convertible notes	(6,128)
Balance as of December 31, 2020	$ —

9. REDEEMABLE CONVERTIBLE PREFERRED STOCK

In August 2016, the Company consummated a financing transaction in which it issued Series A redeemable convertible preferred stock. The issuance of the Series A redeemable convertible preferred stock occurred pursuant to the Series A Preferred Stock Purchase Agreement the Company entered into with certain investors (the “Series A Agreement”). In connection with the issuance of the Series A redeemable convertible preferred stock, certain convertible notes issued by the Company including interest accrued on such notes also converted into 2,333,276 shares of Series A redeemable convertible preferred stock. In connection with the conversion of these notes, the Company recorded a loss on extinguishment of convertible notes of $0.6 million, which is calculated as the difference between the fair value of the Series A redeemable convertible preferred stock and the carrying value of the notes.

At the initial closing of the issuance of the Series A redeemable convertible preferred stock, the Company issued 3,000,731 shares of Series A redeemable convertible preferred stock at $1.22907 per share for a total consideration of $3.7 million. Between November 2016 and November 2017, the Company issued, in aggregate, an additional 955,602 shares of Series A redeemable convertible preferred stock at $1.22907 per share for a total consideration of $1.2 million.

The aggregate purchase price of the Series A redeemable convertible preferred stock was $7.7 million and incurred issuance costs of $0.1 million, recorded as a reduction to Series A redeemable convertible preferred stock carrying value.

In June 2020, the Company entered into a Series B Preferred Stock Purchase Agreement (the “Series B Agreement”) with certain investors in which the Company issued 1,767,401 shares of Series B redeemable convertible preferred stock, $0.0001 par value, for a total consideration of $30.0 million. In connection with the issuance of these shares, the carrying value including accrued interest of the Notes totaling $34.7 million automatically converted into 12,729,434 shares of Series B redeemable convertible preferred stock.

On June 18, 2020, the Company amended its certificate of incorporation in which (i) the Company authorized 15,100,000 of shares of Series B redeemable convertible preferred stock and (ii) the authorized number of Series A redeemable convertible preferred stock was decreased to 6,289,609 shares.

As of each consolidated balance sheet date, the Company's redeemable convertible preferred stock consisted of the following:

	December 31, 2019 (dollars in thousands)				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A preferred stock	6,401,500	6,289,609	$ 7,675	$ 7,730	6,289,609
	6,401,500	6,289,609	$ 7,675	$ 7,730	6,289,609

	December 31, 2020 (dollars in thousands)				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A preferred stock	6,289,609	6,289,609	$ 7,675	$ 7,730	6,407,259
Series B preferred stock	15,100,000	14,496,835	64,387	246,070	14,496,835
	21,389,609	20,786,444	$ 72,062	$ 253,800	20,904,094

As of December 31, 2020, the holders of the Series A redeemable convertible preferred stock and Series B redeemable convertible preferred stock (the "Preferred Stock") have the following rights and preferences:

***Conversion*—**On June 18, 2020, in connection with the conversion of the 2017 Notes, the Company adjusted the conversion price for the Series A redeemable convertible preferred stock of $1.229073 per share to $1.2065. The adjustment was made in accordance with the anti-dilution provisions in the certificate of incorporation then in effect immediately prior to the conversion of the 2017 Notes. The adjustment to the conversion price resulted in neither modification nor extinguishment of the Series A redeemable convertible preferred stock as the terms of the Series A redeemable convertible preferred stock were not amended. The adjustment to the conversion price resulted in additional 117,650 shares of common stock to be issued to holders of the Series A redeemable convertible preferred stock upon conversion of such shares into common stock. As of December 31, 2020, these additional shares of common stock were not issued and outstanding. Each Series B redeemable convertible preferred stock is convertible into an equivalent number of common stock, at any time, at the option of the holder. The initial conversion price is the original issue price.

The conversion price for the Preferred Stock is subject to adjustments for stock splits, stock dividends, or similar recapitalization, and subject to adjustments in accordance with the antidilution provisions.

The shares of Preferred Stock will automatically convert into common shares of the Company immediately upon either (a) the closing of the sale of shares of common stock to the public at a price of at least $50.922231 per share (subject to adjustments for stock dividends, stock split, combination, or other similar recapitalization with respect to the common stock), in a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, resulting in at least $50.0 million of proceeds, net of the underwriting discount and commissions, to the Company or (b) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of a majority of the then outstanding shares of Preferred Stock.

Dividends—Dividends may be paid to the holders of the Series A redeemable convertible preferred stock. The holders the Series A redeemable convertible preferred stock are entitled to

receive non-cumulative dividends at a rate per annum of $0.073744 per share when and if declared by the Board of Directors. The holders of the Series B redeemable convertible preferred stock are entitled to receive a non-cumulative dividend at the rate of 6% per annum of the Series B original issue price per share when and if declared by the Board of Directors. As of December 31, 2019, and 2020, no cash dividends have been declared or paid.

Voting Rights— The holders of the Preferred Stock are entitled to vote on any matter presented to stockholders of the Company for consideration. Each holder of the Preferred Stock will be entitled to cast the number of votes equal to the number of shares of common stock into which the shares of the Preferred Stock held by such holder are convertible on such date.

Redemption—The Preferred Stock does not contain any mandatory redemption features. In accordance with FASB ASC Topic 480, *Distinguishing Liabilities from Equity (ASC 480)*, preferred stock issued with redemption provisions that are outside of the control of the Company or that contain certain redemption rights in a deemed liquidation event is required to be presented outside of stockholders' deficit on the face of the consolidated balance sheet. The Company classified the Preferred Stock outside of the stockholders' deficit as mezzanine equity because in the event of certain deemed liquidation events, which included events such as a sale or merger, that were not solely within the control of the Company, the shares of the Preferred Stock would become redeemable at the option of the holders. As of December 31, 2019 and 2020, the Company did not adjust the carrying values of the Preferred Stock to the redemption values of such shares since a deemed liquidation event did not occur and the shares were not probable of becoming redeemable in the future as of the consolidated balance sheet dates.

Liquidation—In the event of a liquidation, deemed liquidation, dissolution or winding up of the Company, holders of the Preferred Stock will be entitled to be paid out of the assets of the Company that are available for distribution before any payment is made to the holders of common stock. The amount to paid will be the greater of (i) respective original issue prices plus any dividends declared but unpaid or (ii) the amount that would have been payable had all shares of Preferred Stock been converted into common stock immediately before such event. If upon any such liquidation, deemed liquidation, dissolution or winding up of the Company, the assets of the Company available for distribution to its stockholders shall be insufficient to pay the holders of Preferred Stock the full amount to which they shall be entitled, the holders of Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

After the payment of all preferential amounts required to be paid to the holders of Preferred Stock, the remaining assets of the Company available for distribution to its stockholders shall be distributed among the holders of the shares of common stock on a pro rata basis based on the number of shares held by each such holder.

10. STOCKHOLDERS' DEFICIT

Common Stock—Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders provided, however, that, except as otherwise required by law, holders of common stock shall not be entitled to vote on any amendment to the Corporation's Certificate of Incorporation that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the Certificate of Incorporation or pursuant to the Delaware General Corporation Law. Common stockholders are entitled to receive dividends, as may be declared by the Company's Board of Directors, if any, subject

to the preferential dividend rights of the Preferred Stock. No dividends have been declared or paid during the years ended December 31, 2019 and 2020.

At December 31, 2019, the Company did not have sufficient authorized and unissued shares available to satisfy the potential number of shares that would be required to satisfy Series A redeemable convertible preferred stock and stock options into common stock. However, management had the ability and intent to increase authorized shares, which they did on June 16, 2020. At December 31, 2019, the Company adopted the sequencing approach based on the earliest issuance date. Therefore, stock option grants, being the first in priority as the Company established the Company's 2015 Stock Option and Grant Plan (the "2015 Plan") prior to the issuance of the Series A redeemable preferred stock, were allocated sufficient common shares. The Company allocated the remainder of the authorized and unissued shares to the Series A redeemable convertible. This sequencing approach did not change the classification of the Series A redeemable preferred stock nor the stock options.

The Company's common stock available for future issuance is summarized below:

	December 31,	
	2019	2020
Common stock authorized	14,000,000	35,000,000
Common stock issued and outstanding	5,994,246	6,137,206
Common stock authorized and reserved for future issuances:		
Common stock reserved for the conversion of		
Series A redeemable convertible preferred stock	5,117,360	6,289,609
Common stock reserved for issuance upon conversion of		
Series A redeemable convertible preferred stock based on adjustments to the conversion price	—	117,650
Common stock reserved for the conversion of Series B redeemable convertible preferred stock	—	14,496,835
Common stock reserved for the exercise of stock options	2,224,752	3,012,092
Common stock reserved for future issuance of share-based awards	663,642	1,170,692
Total common stock authorized and reserved for future issuance	8,005,754	25,086,878
Unreserved common stock available for future issuance	—	3,775,916

11. STOCK OPTION AND GRANT PLAN

2015 Plan—The Company sponsors the 2015 Plan to encourage and enable the officers, employees, directors, consultants, and other key persons to acquire a proprietary interest in the Company. The 2015 Plan provides for the granting of incentive stock options, non-statutory stock options, and restricted stock awards to eligible employees, officers, directors, consultants, and advisors as determined by the Board of Directors. Terms of restricted stock awards and stock option agreements, including vesting requirements, are determined by the Board of Directors or compensation committee of the Board of Directors, subject to the provisions of the 2015 Plan. The options issued the 2015 Plan expire ten years from the grant date. The options generally vest over four or five years, with 25% vesting on the first anniversary and the balance vesting ratably over the remaining three to four years. Through amendments on December 9, 2015, July 30, 2016, February 15, 2019, and February 26, 2020 the total number of share-based awards authorized for issuance was increased to a total of 4,990,374. As of December 31, 2020, there were 1,170,692 common shares available for future grant under the 2015 Plan.

The Company estimates the fair value of stock option awards on the grant date using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year Ended December 31,	
	2019	2020
Fair value of underlying common stock	$ 0.33	$ 4.30
Risk-free interest rate	2.40%	0.85%
Expected term (in years)	5.97	6.04
Expected volatility	83.01%	81.35%
Dividend yield	0.00%	0.00%

The per share weighted average grant date fair value of stock options granted during the year ended December 31, 2019 and 2020 was $0.26 and $2.95, respectively. As of December 31, 2020, total unrecognized compensation expense related to stock options totaled $3.2 million which is expected to be recognized over a weighted average period of 1.6 years.

The following table summarizes the activity under the Company's stock option activity under the 2015 Plan during the year ended December 31, 2020:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding at January 1, 2020	2,224,752	$ 0.29	8.1	$ 168
Granted	1,125,300	4.3		
Exercised	(142,960)	0.3		
Cancelled or forfeited	(195,000)	1.4		
Outstanding at December 31, 2020	3,012,092	$ 1.73	6.3	$ 15,501
Exercisable at December 31, 2020	1,421,395	$ 0.32	4.5	$ 9,328
Unvested at December 31, 2020	1,590,697	$ 3.00	5.6	$ 6,172

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of options exercised during the years ended December 31, 2019 and 2020, was $0.1 million and $0.4 million, respectively.

There were no restricted shares granted, vested, or forfeited during the year ended December 31, 2020.

The grant date fair value and the stock-based compensation expense related to the vesting of restricted stock under the 2015 Plan was $0 during the years ended December 31, 2019 and 2020.

Stock-Based Compensation Expense—The Company recorded stock-based compensation expense in the following expense categories of its statements of operations and compressive loss:

	Year Ended December 31,	
	2019	2020
	(in thousands)	
Research and development expenses	$ 44	$ 102
General and administrative expenses	64	141
Total stock-based compensation	$ 108	$ 243

12. INCOME TAXES

There was no provision for (benefit from) income taxes for the years ended December 31, 2019 and 2020.

A reconciliation of the Company's effective income tax rate to the U.S. statutory federal income tax rate of 21% for the years ended December 31, 2019 and 2020 is as follows:

	Year Ended December 31,	
	2019	2020
Tax at statutory rate	21.0%	21.0%
State income tax benefit	12.2%	6.4%
Permanent items	(2.1)%	(1.8)%
ASC 740-10 liability	(1.1)%	(0.4)%
Prior year adjustments	16.4%	0.6%
Research and development credit	4.1%	2.4%
Valuation allowance	(50.5)%	(28.2)%
Effective income tax rate	0.0%	0.0%

Deferred tax assets and liabilities reflect the net tax effects of net operating loss and tax credit carryforwards and temporary differences between the carrying amount of assets and liabilities for financial reporting and the amounts used for tax purposes. Significant components of the Company's deferred tax assets and liabilities were as follows for the years ended December 31, 2019 and 2020:

	Year Ended December 31,	
	2019	2020
	(in thousands)	
Deferred tax assets:		
Federal net operating loss carryforwards	$ 2,119	$ 9,824
State net operating loss carryforwards	605	2,837
Capitalized research and development costs	3,053	3,985
Tax credits	852	1,903
Accruals and other	308	545
Total deferred tax assets	$ 6,937	$ 19,094
Valuation allowance	(6,919)	(18,900)
Net total deferred tax assets	$ 18	$ 194
Deferred tax liabilities:		
Prepaid Expenses	(18)	(194)
Total deferred tax liabilities	$ (18)	$ (194)
Net deferred tax assets	$ —	$ —

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. The Company has considered its history of cumulative net losses incurred since inception and has concluded that it is more likely than not that it will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the net deferred tax assets as of December 31, 2020 and 2019. The Company reevaluates the positive and negative evidence at each reporting period.

As of December 31, 2019, and 2020, the Company had federal net operating loss carryforwards of approximately $10.1 million and $46.8 million, respectively, and state net operating loss carryforwards of approximately $9.2 million and $44.9 million, respectively, which are available to reduce future taxable income. Of the $46.8 million federal net operating loss carryforwards, $1.3 million begin to expire in 2034 and the remaining $45.5 million net operating losses carryforward indefinitely. The $44.9 million of Massachusetts net operating loss carryforwards begin to expire in 2034. As of December 31, 2019, and 2020, the Company also had federal tax credits of $0.7 million and $1.6 million, respectively, and state tax credits of $0.4 million and $0.7 million, respectively. The tax credit carryforwards will expire at various dates beginning in 2029.

The 2017 Tax Cuts and Jobs Act (“TCJA”) will generally allow losses incurred after 2017 to be carried over indefinitely but will generally limit the net operating loss deduction to the lesser of the net operating loss carryover or 80% of a corporation’s taxable income (subject to Section 382 of the Internal Revenue Code of 1986, as amended). Also, there will be no carryback for losses incurred after 2017. Losses incurred prior to 2018 will generally be deductible to the extent of the lesser of a corporation’s net operating loss carryover or 100% of a corporation’s taxable income and be available for twenty years from the period the loss was generated. On March 27, 2020, the Coronavirus Aid, Relief and Economic Security Act (the “CARES Act”) was passed by the U.S. Congress and signed into United States law. The CARES Act temporarily allows the Company to carryback federal net operating losses arising in 2018, 2019, and 2020 to each of the five taxable years preceding the taxable year in which the loss arises. The net operating losses generated in these years could fully offset prior year taxable income without the 80% taxable income limitation under the TCJA. Additionally, the CARES Act temporarily suspends the 80% taxable income limitation, allowing the net operating losses carryforward to fully offset taxable income in tax years beginning before January 1, 2021

The utilization of the net operating loss carryforwards and research and development tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended (the “Code”) due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. The Company has not conducted a formal study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382 and 383 of the Code, at any time since inception, utilization of the net operating loss carryforwards or research and development tax credit carryforwards may be subject to an annual limitation under Section 382 and 383 of the Code, which is determined by first multiplying the value of the Company’s stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization.

The Company has not yet conducted a study of research and development credit carryforward. Such a study, once undertaken by the Company, may result in an adjustment to the Company’s research & development credit carryforward. A full valuation allowance has been provided against the

Company's research and development credit and, if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the balance sheet or statement of operations if an adjustment is required.

ASC 740 requires a valuation allowance to reduce the deferred tax assets reported if, based on the weight of the evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. After consideration of all the evidence, both positive and negative, the Company has recorded a valuation allowance against its deferred tax assets as of December 31, 2019 and 2020, because the Company's management has determined that it is more likely than not that these assets will not be fully realized. The increase in the valuation allowance recoded during the year primarily relates to the net operating loss incurred by the Company as well as the increase in research and development credits.

The following table reflects the roll-forward of the Company's valuation allowance:

	Year Ended December 31,	
	2019	2020
	(in thousands)	
Valuation allowance at beginning of year	$2,885	$ 6,919
Increases recorded to income tax provision	4,034	11,981
Valuation allowance at end of year	$6,919	$18,900

The Company accounts for Uncertainty in Income Taxes under the provisions of ASC 740 which defines the thresholds for recognizing the benefits of tax return positions in the consolidated financial statements as "more likely than not" to be sustained by the taxing authority. The tax benefit is measured based on the largest benefit that has a greater than 50% likelihood of being realized upon ultimate settlement. As of December 31, 2019, and 2020, the Company has recorded an unrecognized tax benefit of $0.2 million and $0.3 million, respectively.

	Year Ended December 31,	
	2019	2020
	(in thousands)	
Balance at beginning of the period	$—	$160
Settlement/decreases related to tax positions taken during prior years	—	(20)
Increases related to tax positions taken during prior years	54	—
Increases related to tax positions taken during the current year	106	209
Balance at end of the period	$160	$349

The Company has reviewed the tax positions taken, or to be taken, in its tax returns for all tax years currently open to examination by a taxing authority. Unrecognized tax benefits represent the aggregate tax effect of differences between tax return positions and the benefits recognized in the consolidated financial statements. As of December 31, 2019, and 2020, the Company had $0.2 million and $0.3 million respectively, of net unrecognized tax benefits, related to tax credit carryforwards. The Company does not expect the amount of unrecognized tax benefits to change over next 12 months. The Company accrues interest and penalties related to unrecognized tax benefits as a component of its "Provision for (benefit from) income taxes." The Company did not recognize any interest or penalties related to uncertain tax positions during the two years ended December 31, 2020.

The Company files U.S. federal and state income tax returns in various jurisdictions. The tax filings related to the Company's federal and state taxes are currently open to examination for tax years

2017 through 2020. There are currently no federal or state audits in process. In addition, the Company generated federal and state research and development tax credits in tax year 2016 which would subject this year to examination when the credits are utilized in a future year. There are currently no federal or state examinations in progress.

In March 2020, the CARES Act was signed into law. The Company considered these provisions under the CARES Act and concluded that it did not have a material impact to the Company's results of operations, cash flows and consolidated financial statements.

13. NET LOSS PER SHARE

Net Loss per Share Attributable To Common Stockholders—Because the Company reports a net loss attributable to common stockholders, basic and diluted net loss per share attributable to common stockholders are the same for both years presented. All preferred stock and stock options have been excluded from the computation of diluted weighted-average shares outstanding because such securities would have an antidilutive impact. The following common stock equivalents outstanding as of December 31, 2019 and 2020 have been excluded from the calculation of diluted net loss per share because their inclusion would have been antidilutive:

	December 31,	
	2019	2020
Options to purchase common stock	2,224,752	3,012,092
Redeemable convertible preferred stock	6,289,609	20,904,094
Convertible notes	10,870,123	—

14. RELATED PARTY TRANSACTIONS

Convertible Notes

In connection with the issuance of the 2017 Notes, the 2018 Notes and 2020 Notes, the Company issued, in aggregate, $16.5 million of convertible promissory Notes to ALS Invest 1 B.V, Morningside Ventures Investments Limited, and certain members of the board of directors of the Company. ALS Invest 1. B.V and Morningside Ventures Investments Limited are each a 5% significant stockholder and have appointed representatives to the board of directors of the Company. ALS Invest 1 B.V and Morningside Ventures Investments Limited have each designated a member to the board of directors of the Company. These notes were issued under the same terms and conditions as the 2018 Notes and 2020 Notes (See Note 6).

Employment Agreements

In July 2015, the Company entered into employment agreements, as subsequently amended in April 2021, with Joshua Cohen and Justin Klee. Joshua Cohen and Justin Klee are co-founders of the Company and both are currently Co-Chief Executive Officer (Co-CEO) of the Company. The employment agreements with Mr. Cohen and Mr., Klee provide an annual base salary, cash bonus, a stock option bonus, and the ability to participate in the employee benefit plans. The employment agreements with Mr. Cohen and Mr. Klee are at-will employment.

15. COMMITMENTS AND CONTINGENCIES

Operating Leases—In October 2018, the Company entered into a lease agreement ("Original Lease") for its office space in Cambridge, Massachusetts. The lease commenced in December 2018 and was set to expire in December 2023. The Original Lease did not include an option to renew at the

end of the term. The Original Lease called for a security deposit of less than $0.1 million. The annual rent was subject to fixed annual increases. The security deposit is included in other assets on the consolidated balance sheet as of December 31, 2019. The Company analyzed the terms of the Original Lease and determined that it was an operating lease (see Note 2).

In January 2020, the Company entered into an amendment ("Lease Amendment") to extend the lease term of the Original Lease and to lease an additional office space ("Expansion Space"). The extension of the term of the Original Lease and the lease term for the Expansion Space is six years from the date the Expansion Space was delivered to the Company, which occurred in October 2020. As a result of the Lease Amendment, the lease term of the Original Lease and the Expansion Space will expire in October 2026.

Rent expense, including common area maintenance, parking and other rental fees for the years ended December 31, 2019 and 2020, was $0.1 million and $0.2 million, respectively.

Future minimum payments under the noncancelable operating lease as of December 31, 2020, are as follows:

	Minimum Lease Payments (in thousands)
Years Ending December 31,	
2021	$ 523
2022	532
2023	541
2024	555
2025	563
Thereafter	497
	$ 3,211

***Letter of Credit*—**As of December 31, 2019, the Company had paid a security deposit of less than $0.1 million to the landlord in connection with the Original Lease, which was set to expire in 2023. In connection with the Lease Amendment, the Company received back the initial security deposit and issued a security deposit in the form of a letter of credit to the landlord in the amount of $0.2 million. The security deposit will mature in 2026. The letter of credit is collateralized by restricted cash and has been classified as such on the consolidated balance sheet as of December 31, 2020.

***Legal Proceedings*—**The Company is not currently a party to any material legal proceedings. At each reporting date, the Company evaluates whether or not a potential loss amount or potential range of loss is probable and reasonably estimated under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company recognizes expenses for its costs related to its legal proceedings, as incurred.

***Royalty Payments*—**Between August 2016 and February 2019, the Company entered into grant agreements with the Grantors. Under the terms of the grant agreements, the Company was granted, in aggregate, $4.3 million in grants. These grants were provided to the Company for the purpose of furthering the research and development of AMX0035 as a therapeutic benefit for ALS disease and Alzheimer's diseases. Under the terms of the arrangements, the Company would receive tranche of funds as it completes certain milestones. Pursuant to the terms of the grant agreements, the Company has certain payment obligations that are contingent upon future events such as the achievement of commercialization or the receipt of proceeds from a revenue generating transaction resulting from the projects for which the grants are used for.

Pursuant to the terms of the respective grant agreements among the Company, ALS Association and ALS Finding a Cure, the Company will be required to make royalty payments to each Grantor in the total amount equal to 150% of the grant received. The royalty payments will be achieved through a combination of the following payment methods: (i) an annual installment payment of 3% of net sales of any products developed under the project for which the grant was used for and (ii) 3% of cash proceeds resulting from revenue generating transaction under the project for which the grants are used for.

Under the terms of the respective grant agreements among the Company, Alzheimer's Drug Discovery Foundation, the Alzheimer's Association, and Cure Alzheimer's Fund, the Company will make royalty payments up to the maximum amount of $15.0 million to each Grantor (or $45.0 million in aggregate). The royalty payment will be made through a combination of the following payment methods: (i) 4% of annual net sales of any product commercialized from the project for which the grant was used for and directly related to the treatment of the Alzheimer's disease and (ii) 15% of all royalties and cash proceeds resulting from revenue generated transactions associated with the projects for which the grants were used for under the grant agreements. As the achievement and timing of these future royalty payments are not probable or estimable, such amounts have not been included in the consolidated balance sheets as of December 31, 2019 and 2020.

16. SUBSEQUENT EVENTS

As of December 31, 2020, and for the year then ended, the Company evaluated subsequent events through April 26, 2021, the date on which these consolidated financial statements were available for issuance.

***2021 Notes*—**In January and February 2021, the Company received $27.3 million from the issuance of convertible notes due on June 30, 2022.

***PPP Loan*—**The Company applied for forgiveness of the PPP Loan. In March 2021, the PPP Loan was forgiven.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands, except share and per share amounts)
(unaudited)

	December 31, 2020	September 30, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 12,877	$ 76,677
Short-term investments	—	49,025
Prepaid expenses and other current assets	762	2,524
Deferred offering costs	—	1,716
Total current assets	13,639	129,942
Property and equipment, net	151	329
Restricted cash	189	189
Other assets	125	—
Total assets	$ 14,104	$ 130,460
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Deficit		
Current liabilities:		
Accounts payable	$ 3,613	$ 3,026
Accrued expenses	3,713	12,120
Total current liabilities	7,326	15,146
Deferred rent	14	31
Accrued interest	2	2
PPP Loan	263	—
Proceeds received in advance of issuance of 2021 Notes	1,162	—
Total liabilities	8,767	15,179
Commitments and contingencies (Note 13)		
Series A redeemable convertible preferred stock, $0.0001 par value; 6,289,609 shares authorized as of December 31, 2020 and September 31, 2021; 6,289,609 shares issued and outstanding as of December 31, 2020 and September 30, 2021, respectively; aggregate liquidation preference of $7,730	7,675	7,675
Series B redeemable convertible preferred stock, $0.0001 par value; 15,100,000 shares authorized as of December 31, 2020 and September 30, 2021; 14,496,835 shares issued and outstanding as of December 31, 2020 and September 30, 2021; aggregate liquidation preference of $246,070	64,387	64,387
Series C-1 redeemable convertible preferred stock, $0.0001 par value; 0 and 13,150,430 shares authorized as of December 31, 2020 and September 30, 2021, respectively; 0 and 13,150,430 shares issued and outstanding as of December 31, 2020 and September 30, 2021, respectively; aggregate liquidation preference of $0 and $135,000 as of December 31, 2020 and September 30, 2021, respectively	—	134,791
Series C-2 redeemable convertible preferred stock, $0.0001 par value; 0 and 3,170,585 shares authorized as of December 31, 2020 and September 30, 2021, respectively; 0 and 3,170,585 shares issued and outstanding as of December 31, 2020 and September 30, 2021, respectively; aggregate liquidation preference of $0 and $27,666 as of December 31, 2020 and September 30, 2021, respectively	—	32,498
Stockholders' deficit:		
Common stock, $0.0001 par value; 35,000,000 and 56,500,000 shares authorized as of December 31, 2020 and September 30, 2021, respectively; 6,137,206 and 6,799,157 shares issued and outstanding as of December 31, 2020 and September 30, 2021, respectively	1	1
Additional paid-in capital	1,188	3,431
Accumulated deficit	(67,914)	(127,501)
Accumulated other comprehensive loss	—	(1)
Total stockholders' deficit	(66,725)	(124,070)
Total liabilities, redeemable convertible preferred stock and stockholders' deficit	$ 14,104	$ 130,460

The accompanying notes are an integral part of these condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(Amounts in thousands, except share and per share amounts)
(unaudited)

	Nine Months Ended September 30,	
	2020	2021
Grant revenue	$ 300	$ 285
Operating expenses:		
Research and development	19,581	30,646
General and administrative	11,132	24,012
Total operating expenses	30,713	54,658
Loss from operations	(30,413)	(54,373)
Other income (expense), net:		
Interest income	14	6
Interest expense	(2,287)	—
Change in fair value of derivative liability	(1,270)	—
Change in fair value of convertible notes	—	(5,228)
Other income, net	268	8
Total other expense, net	(3,275)	(5,214)
Net loss	(33,688)	(59,587)
Net loss per share attributable to common stockholders —basic and diluted	$ (5.55)	$ (9.20)
Weighted-average shares used in computing net loss per share attributable to common stockholders—basic and diluted	6,069,726	6,477,140

The accompanying notes are an integral part of these condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(Amounts in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2020	2021
Net loss	$(33,688)	$(59,587)
Other comprehensive income (loss):		
Foreign currency translation adjustment, net of tax of $0 for the nine months ended September 30, 2020 and 2021	—	4
Unrealized loss on short-term investments, net of tax of $0 for the nine months ended September 30, 2020 and 2021	—	(5)
Other comprehensive loss	—	(1)
Comprehensive loss	$(33,688)	$(59,588)

The accompanying notes are an integral part of these condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(Amounts in thousands, except share amounts)
(unaudited)

	Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Series C-1 Redeemable Convertible Preferred Stock		Series C-2 Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance as of December 31, 2019	6,289,609	$ 7,675	—	$ —	—	$ —	—	$ —	5,994,246	$ 1	$ 276	$ —	$ (25,634)	$ (25,357)
Issuance of Series B redeemable convertible preferred stock, net of issuance costs of $35	—	—	1,767,401	29,958	—	—	—	—	—	—	—	—	—	—
Conversion of convertible notes and accrued interest into Series B redeemable convertible preferred stock	—	—	12,729,434	34,429	—	—	—	—	—	—	—	—	—	—
Recognition of contingent beneficial conversion feature	—	—	—	—	—	—	—	—	—	—	621	—	—	621
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	99,382	—	32	—	—	32
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	122	—	—	122
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(33,688)	(33,688)
Balance as of September 30, 2020	6,289,609	$ 7,675	14,496,835	$ 64,387	—	$ —	—	$ —	6,093,628	$ 1	$ 1,051	$ —	$ (59,322)	$ (58,270)

	Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Series C-1 Redeemable Convertible Preferred Stock		Series C-2 Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	T Stock D
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance as of December 31, 2020	6,289,609	$ 7,675	14,496,835	$ 64,387	—	$ —	—	$ —	6,137,206	$ 1	$ 1,188	$ —	$ (67,914)	$
Issuance of Series C-1 redeemable convertible preferred stock, net of issuance costs of $209	—	—	—	—	13,150,430	134,791	—	—	—	—	—	—	—	
Conversion of convertible notes and accrued interest into Series C-2 redeemable convertible preferred stock, net of issuance costs of $50	—	—	—	—	—	—	3,170,585	32,498	—	—	—	—	—	
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	661,951	—	198	—	—	
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	2,045	—	—	
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—		(1)	—	
Net loss	—	—	—	—	—	—	—	—	—	—		—	(59,587)	
Balance as of September 30, 2021	6,289,609	$ 7,675	14,496,835	$ 64,387	13,150,430	$134,791	3,170,585	$ 32,498	6,799,157	$ 1	$ 3,431	$ (1)	$ (127,501)	$

The accompanying notes are an integral part of these condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(Amounts in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2020	2021
Cash flows used in operating activities:		
Net loss	$(33,688)	$ (59,587)
Adjustments to reconcile net loss to net cash used in operating activities:		
Change in fair value of derivative liability	1,270	—
Non-cash interest expense	1,738	—
Stock-based compensation expense	122	2,045
Depreciation expense	—	30
Net amortization of premiums and discounts on investments	—	23
Gain on extinguishment of convertible notes	(268)	—
Change in fair value of convertible notes	—	5,228
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(476)	(1,764)
Other assets	(125)	125
Accounts payable	952	(716)
Accrued expenses and deferred rent	2,118	8,061
Accrued interest and accrued interest—related parties	548	—
Net cash used in operating activities	(27,809)	(46,555)
Cash flows used in investing activities:		
Purchases of property and equipment	—	(167)
Purchases of investments	—	(49,053)
Net cash used in investing activities	—	(49,220)
Cash flows from financing activities:		
Proceeds from PPP Loan	263	—
Proceeds from issuance of convertible notes—related parties	4,794	14,272
Proceeds from issuance of convertible notes, net of issuance costs	10,598	11,887
Issuance costs related to conversion of convertible notes	—	(50)
Proceeds from issuance of Series B redeemable convertible preferred stock, net of issuance costs	29,958	—
Proceeds from issuance of Series C-1 redeemable convertible preferred stock, net of issuance costs	—	134,791
Proceeds from exercise of stock options	32	198
Payment of deferred offering costs	—	(1,527)
Net cash provided by financing activities	45,645	159,571
Effect of exchange rate changes on cash, cash equivalents and restricted cash	—	4
Net increase in cash, cash equivalents and restricted cash	17,836	63,800
Cash, cash equivalents and restricted cash, beginning of period	3,091	13,066
Cash, cash equivalents and restricted cash, end of period	$ 20,927	$ 76,866
Supplemental disclosure of cash flow information:		
Recognition of initial derivative liability and associated debt discount	$ 4,636	$ —
Conversion of convertible notes into Series B redeemable convertible preferred stock	$ 34,697	$ —
Conversion of convertible notes and accrued interest into Series C-2 redeemable convertible preferred stock	$ —	$ 32,548
Unrealized loss on short-term investments	$ —	$ (5)
Purchases of property and equipment included in accounts payable	$ —	$ 41
Deferred offering costs included in accounts payable and accrued expenses	$ —	$ 189

The accompanying notes are an integral part of these condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(unaudited)

1. NATURE OF BUSINESS

Amylyx Pharmaceuticals, Inc. ("Amylyx") was incorporated under the laws of the State of Delaware on January 10, 2014. In October 2020 and August 2021, Amylyx created wholly owned subsidiaries, Amylyx Pharmaceuticals Canada, Inc. ("Amylyx Canada") in Calgary, Canada, and Amylyx Pharmaceuticals EMEA B.V. in Amsterdam, the Netherlands ("Amylyx EMEA", and collectively with "Amylyx Canada" and "Amylyx", the "Company"). As of September 30, 2021, Amylyx EMEA did not have operations. The Company is headquartered in Cambridge, Massachusetts. The Company is a clinical stage biotechnology company with a goal to improve the quality and length of life of patients suffering from neurodegenerative disease. The Company is pursuing commercialization of its asset, AMX0035, which it believes is the first therapeutic to show both a functional and survival benefit in a large-scale clinical trial of patients with amyotrophic lateral sclerosis, or ALS. The Company believes AMX0035 has the potential to be a foundational therapy across a broad range of neurodegenerative diseases. The Company has designed AMX0035 to target two key pathways of neuron death, specifically endoplasmic reticulum, or ER, stress and mitochondrial dysfunction. The Company is focused on the development of and potential commercialization of AMX0035 for ALS globally. In addition, the Company is developing AMX0035 for other neurodegenerative diseases by leveraging its unique knowledge and relationships in the neurodegenerative space.

Risks and Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, the outcome of clinical trials, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, ability to secure additional capital to fund operations, and risks associated with the COVID-19 global pandemic, including potential delays associated with the Company's ongoing and anticipated trials. COVID-19 may have an adverse impact on the Company's operations, supply chains and distribution systems or those of its contractors, and increase expenses, including as a result of impacts associated with preventive and precautionary measures that are being taken, such as restrictions on travel and border crossings, quarantine polices and social distancing. The Company and its contractors may experience disruptions in supply of items that are essential for its research and development activities, including, for example, raw materials and bulk drug substances that the Company imports from Europe and Canada used in the manufacturing of AMX0035, and any future product candidates. In addition, the spread of COVID-19 has disrupted global healthcare and healthcare regulatory systems which could divert healthcare resources away from, or materially delay, U.S. Food and Drug Administration ("FDA") approval and approval by other health authorities worldwide with respect to AMX0035 and any future product candidates. Furthermore, the Company's clinical trials may be negatively affected by the COVID-19 outbreak. Site initiation, patient enrollment and patient follow-up visits may be delayed, for example, due to prioritization of hospital resources toward the COVID-19 outbreak, travel restrictions, the inability to access sites for initiation and monitoring, and difficulties recruiting or retaining patients in the Company's ongoing and future clinical trials.

There can be no assurance that the Company will be able to successfully complete the development of, or receive regulatory approval for, any products developed, and if approved, that any products will be commercially viable. Any products resulting from the Company's current research and development efforts will require significant additional research and development, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts will require significant amounts of additional capital, adequate personnel, infrastructure, and extensive compliance reporting capabilities. The Company has not generated any revenues from the sale of any

products to date. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Going Concern

In accordance with Accounting Standards Update ("ASU") 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are conditions and events that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the condensed consolidated financial statements are issued.

Since its inception, the Company has devoted substantially all of its efforts to research and development activities, including recruiting management and technical staff, raising capital, producing materials for non-clinical and clinical studies, and building infrastructure to support such activities. Expenses have primarily been for research and development and related general and administrative costs. The Company has generated revenues through five grants from ALS Association, ALS Finding a Cure Foundation, Cure Alzheimer's Fund, Alzheimer's Drug Discovery Foundation and Alzheimer's Association (collectively, the "Grantors"). In addition to money received from its grants, the Company has also financed its operations through the issuance of redeemable convertible preferred stock and convertible notes (see Notes 8 and 6, respectively). In addition, the Company has financed its operations pursuant to the loan under the Paycheck Protection Program, or the PPP, of the Coronavirus Aid, Relief, and Economic Security ("CARES") Act (the "PPP Loan") as administered by the Small Business Administration ("SBA"). In October 2021, the Company repaid the outstanding balance of its PPP Loan in full (see Note 14).

The accompanying condensed consolidated financial statements have been prepared on a going-concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The Company has incurred recurring losses and negative cash flows from operations since inception. As of September 30, 2021, the Company had an accumulated deficit of $127.5 million. The Company expects its operating losses and negative operating cash flows to continue into the foreseeable future as it continues to build capabilities and develop AMX0035, and any future product candidates. These conditions raise substantial doubt regarding the Company's ability to continue as a going concern within one year of the issuance date of the condensed consolidated financial statements. The Company's plans to address the capital shortfall include negotiating additional equity financing and alternative sources of financial support and considering cost containment efforts. Due to the uncertainty inherent in these efforts, the Company has concluded that substantial doubt exists with respect to its ability to continue as a going concern for at least the next twelve months from the date these condensed consolidated financial statements are issued.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation and Consolidation—The accompanying unaudited condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and include the accounts of the Company and its wholly owned subsidiaries, Amylyx Canada and Amylyx EMEA, after elimination of all intercompany accounts and transactions. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and ASU of the Financial Accounting Standards Board ("FASB").

Unaudited interim condensed financial information—The accompanying condensed consolidated balance sheet as of September 30, 2021, condensed consolidated statements of operations, condensed consolidated statements of comprehensive loss, condensed consolidated

statements of redeemable convertible preferred stock and stockholders' deficit, and condensed consolidated statements of cash flows for the nine months ended September 30, 2020 and 2021, are unaudited. The condensed consolidated balance sheet as of December 31, 2020 was derived from audited annual financial statements but does not contain all of the footnote disclosures from the annual financial statements.

The accompanying condensed consolidated financial statements have been prepared on the basis consistent with the audited annual financial statements as of and for the year ended December 31, 2020, and reflect all adjustments which are, in the opinion of management, necessary for the fair presentation of the Company's financial position as of September 30, 2021, and the condensed results of its operations and its cash flows for the nine months ended September 30, 2020 and 2021. All such adjustments made to the condensed consolidated financial statements are normal and recurring in nature. The financial data and other information disclosed in these notes related to the nine months ended September 30, 2020 and 2021 are also unaudited. The condensed consolidated results of operations for the nine months ended September 30, 2021 are not necessarily indicative of the results to be expected for the full year ending December 31, 2021 or any other period. These condensed consolidated financial statements should be read in conjunction with the Company's audited financial statements included elsewhere in this prospectus.

During the nine months ended September 30, 2021, there were no other significant changes to the Company's significant accounting policies as described in the Company's audited condensed consolidated financial statements as of and for the year ended December 31, 2020 except as described below.

Use of Estimates—The preparation of the condensed consolidated financial statements in conformity with GAAP requires the Company's management to make estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the financial statements and the reported amount of expenses during the reporting periods. Actual results could differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies in developing the estimates and assumptions that are used in the preparation of the condensed consolidated financial statements. Management must apply significant judgment in this process. Management's estimation process often may yield a range of potentially reasonable estimates and management must select an amount that falls within that range of reasonable estimates. Estimates are used in the following areas, among others: determining the fair value of the Company's common stock; determining the fair value of derivative liabilities; determining the fair value of convertible notes, accrued expenses; stock option valuations; valuation of short-term investments; valuation allowance for deferred tax assets and research and development expenses.

Restricted Cash—As of December 31, 2020 and September 30, 2021, the Company maintained a restricted cash account with a balance of $0.2 million. The restricted cash represents collateral provided for a letter of credit issued as a security deposit in connection with the Company's lease of its corporate office. The lease expires in October 2026 at which time the cash will be released from restriction.

Short-Term Investments—Short-term investments are composed of corporate debt securities and commercial paper with maturities of less than one year from the balance sheet date. The Company classifies all of its short-term investments as available-for-sale. Accordingly, these investments are recorded at fair value, which is determined based on quoted market prices. Unrealized gains and losses on available-for-sale securities are included as a separate component of other accumulated comprehensive loss. The cost of short-term investments is adjusted for amortization of premiums and accretion of discounts until maturity. Such amortization and accretion are included in other income, net. Realized gains and losses are included in other income, net. The Company

evaluates short-term investments for other-than-temporary impairment at the balance sheet date. Declines in fair value, if any, determined to be other than temporary-than-temporary are also included in other income, net.

When assessing short-term investments for other-than-temporary declines in value, the Company considers such factors as, among other things, how significant the decline in value is as a percentage of the original cost, how long the market value of the investment has been less than its original cost, and the Company's ability and intent to retain the investment for a period of time sufficient to allow for any anticipated recovery in fair value and market conditions in general. As of September 30, 2021, there were no impairment charges on short-term investments.

Fair Value Option—As permitted under ASC 825, *Financial Instruments*, ("ASC 825") the Company elected the fair value option to account for the 2021 Notes (as defined in Note 6). In accordance with ASC 825, the Company recorded the 2021 Notes at fair value with changes in fair value recorded in the condensed consolidated statement of operations for the nine months ended September 30, 2021. As a result of applying the fair value option, direct costs and fees related to the 2021 Notes were expensed as incurred. The Company concluded it was appropriate to apply the fair value option to the 2021 Notes because they are liabilities that are not, in whole or in part, classified as a component of stockholders' deficit. In addition, the 2021 Notes met other applicable criteria for electing fair value option under ASC 825.

Deferred Offering Costs—The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings, including the initial public offering (IPO), as deferred costs until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' deficit as a reduction of proceeds generated as a result of the offering. Should an in-process equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the condensed consolidated statements of operations and condensed consolidated statements of comprehensive loss. The Company recorded deferred offering costs of $1.7 million, which are included in the condensed consolidated balance sheet as of September 30, 2021.

Property and Equipment, net—Property and equipment are stated at cost, net of accumulated depreciation. Depreciation of property and equipment is calculated using the straight-line method over the estimated useful lives of the respective assets. Maintenance and repairs that do not improve or extend the life of the assets are expensed when incurred. Upon sale or retirement of assets, the cost and accumulated depreciation are removed from the condensed consolidated balance sheets and any resulting gain or loss is reflected in the condensed consolidated statements of operations and condensed consolidated statements of comprehensive loss in the period realized. The range of useful lives of property and equipment is as follows:

	Estimated Useful Life
Leasehold improvements	Lesser of the estimated life or remaining lease term
Furniture and fixtures	4 years
Computer hardware and software	3 years
Construction in progress	Not depreciated

Foreign Currency Translation—The functional currency of the Company is the U.S. dollar. The functional currency of the Company's foreign subsidiaries is the applicable local currency. Assets and liabilities denominated in foreign currencies are translated into U.S. dollars, the reporting currency, at the exchange rate prevailing at the balance sheet date. Income and expenses denominated in foreign currencies are translated into U.S. dollars at the average exchange rate for the period. Adjustments

from foreign currency translation, net of tax are included as a separate component of other comprehensive loss in the condensed consolidated statements of comprehensive loss.

Fair Value Measurements—Assets and liabilities recorded at fair value on a recurring basis in the condensed consolidated balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

- ***Level 1***—Quoted prices in active markets for identical assets or liabilities.
- ***Level 2***—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- ***Level 3***—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The Company's financial instruments consist of cash, cash equivalents, restricted cash, accounts receivable, short-term investments, accounts payable, accrued expenses and convertible notes. The Company's short-term investments are carried at fair value, determined according to Level 2 inputs to the fair value hierarchy described above. The Company's 2021 Notes (as defined in Note 6) and derivative liability is carried at fair value, determined according to Level 3 inputs in the fair value hierarchy described above (see Note 7). The remaining financial instruments are stated at their respective carrying amounts, which approximate fair value due to the short-term nature of these assets and liabilities.

Recent Accounting Pronouncements

New Accounting Pronouncements Not Yet Adopted—In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* ("ASC 842"), which sets out the principles for the recognition, measurement, presentation, and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, for finance and operating leases, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. ASC 842 provides a lessee with an option to not account for leases with a term of 12 month or less as leases in the scope of the new standard. ASC 842 supersedes the previous leases standard, ASC 840 *Leases*. In July 2018, the FASB issued supplemental adoption guidance and clarification to ASC 842 within

ASU No. 2018-10, *Codification Improvements to Topic 842, Leases* and ASU No. 2018-11, *Leases (Topic 842): Targeted Improvements*. ASU No. 2018-11 provides another transition method in addition to the existing modified retrospective transition method by allowing entities to initially apply the new leasing standard at the adoption date and recognize a cumulative-effect adjustment to the opening balance of accumulated deficit in the period of adoption. In July 2019, the FASB delayed the effective date for this ASU for non-public entities (including emerging growth companies), and this ASU is effective for fiscal years beginning after December 15, 2021 and interim periods within fiscal years beginning after December 15, 2022, with early adoption permitted. The ASU is effective for the Company on January 1, 2022 and the Company intends to adopt the ASU when it becomes effective. The Company is currently evaluating the impact the adoption of these ASUs will have on its consolidated financial statements and related disclosures. The standard is expected to have a material impact on the consolidated balance sheet related to the recognition of right-of-use assets and lease liabilities for operating leases. The standard is not expected to have a material impact on the consolidated statement of operations.

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326) Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"), which requires an entity to utilize a new impairment model known as the current expected credit loss model to estimate its lifetime "expected credit loss" and record an allowance that, when deducted from the amortized cost basis of the financial assets and certain other instruments, including but not limited to available-for-sale debt securities. Credit losses relating to available-for-sale debt securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. ASU 2016-13 requires a cumulative effect adjustment to the consolidated balance sheet as of the beginning of the first reporting period in which the guidance is effective. In November 2019, the FASB issued ASU 2019-10, *Financial Instruments—Credit Losses (Topic 326), Derivatives and Hedging (Topic 815) and Leases (Topic 842): Effective Dates*, which defers the effective date of ASU 2016-13 to fiscal years beginning after December 15, 2022 for all entities except Securities and Exchange Commission filers that are not smaller reporting companies. ASU 2016-13 will be effective for the Company beginning January 1, 2023. The Company intends to adopt the ASU when it becomes effective. The Company is currently evaluating the impact of this ASU on its consolidated financial statements and related disclosures.

Recently Adopted Accounting Pronouncements—In August 2020, the FASB issued ASU 2020-06, *Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging-Contracts in Entity's Own Equity* (*Subtopic 815-40*). ASU 2020-06 simplifies the accounting for certain financial instruments with characteristics of liabilities and equity, including convertible instruments and contracts on an entity's own equity. ASU 2020-06 removes from U.S. GAAP the separation models for (i) convertible debt with a cash conversion feature and (ii) convertible instruments with a beneficial conversion. In addition, the new guidance amends the accounting for certain contracts in an entity's own equity that are currently accounted for as derivatives because of specific settlement provisions. Also, the new guidance requires entities to apply the if-converted method to all convertible instruments for calculation of diluted earnings per share and the treasury stock method is no longer available.

The Company early adopted ASU 2020-06 on January 1, 2021 using the modified retrospective transition approach. The adoption of this guidance did not have a material impact on the Company's condensed consolidated financial statements.

3. SHORT-TERM INVESTMENTS

Short-term investments, which are classified as available-for-sale, consisted of the following:

	September 30, 2021		
	Amortized Cost Basis	Unrealized Loss	Fair Values
	(in thousands)		
Commercial paper	$ 33,964	$ —	$33,964
Corporate debt securities	15,066	(5)	15,061
Total short-term investments	$ 49,030	$ (5)	$49,025

4. PROPERTY AND EQUIPMENT, NET

Property and equipment, net consisted of the following:

	December 31, 2020	September 30, 2021
	(in thousands)	
Furniture and fixtures	$ 49	$ 88
Computer hardware and software	—	45
Leasehold improvements	41	51
Construction in progress	62	176
Total property and equipment	152	360
Less: accumulated depreciation	(1)	(31)
Total property and equipment, net	$ 151	$ 329

5. ACCRUED EXPENSES

Accrued expenses consisted of the following:

	December 31, 2020	September 30, 2021
	(in thousands)	
External research and development	$ 293	$ 6,813
Payroll and employee related expenses	1,855	2,013
Accrued legal and other professional fees	1,565	3,031
PPP Loan	—	263
Total accrued expenses	$ 3,713	$ 12,120

In April 2020, the Company obtained a PPP Loan from First Republic Bank in the aggregate amount of $0.3 million, which was established under the CARES Act. Interest accrues on the PPP Loan beginning with the initial disbursement. The Company has determined to account for the PPP Loan as debt under ASC 470, *Debt*. The PPP Loan had an outstanding balance of $0.3 million as of December 31, 2020 and September 30, 2021. The outstanding balance of the PPP Loan is included in accrued expenses in the condensed consolidated balance sheet as of September 30, 2021.

In March 2021, the PPP Loan was forgiven in full. Notwithstanding the forgiveness, the Company intends to repay it, and has recorded the principal and accrued interest in its condensed consolidated balance sheets. See Note 14, *Subsequent Events*, to these notes to condensed consolidated financial statements for additional information.

6. CONVERTIBLE NOTES

Convertible notes

There were no convertible notes outstanding as of December 31, 2020 and September 30, 2021.

Issuance of the 2017 Notes, 2018 Notes, 2019 Notes and 2020 Notes (collectively, the "Notes")

In July 2017, the Company issued $2.3 million convertible notes ("2017 Notes") to certain investors with a maturity date of December 31, 2021. The 2017 Notes were secured and carried an interest rate of 6%.

In November 2018, the Company issued $13.0 million convertible notes ("2018 Notes") with a maturity date of December 31, 2021. The 2018 Notes were secured and carried an interest rate of 6%.

In December 2019, the Company issued $0.6 million of convertible notes ("2019 Notes") to certain investors with a maturity date of December 31, 2021. The 2019 Notes were secured and carried an interest rate of 2%.

In January, February and April 2020, the Company issued, in aggregate, $15.4 million in convertible notes ("2020 Notes") to certain investors with a maturity date of December 31, 2021. The 2020 Notes were secured and carried an interest rate of 2%.

Embedded Derivatives

The Company assessed all the terms of the Notes in order to identify any potential embedded features and determined that the redemption features (which are discussed in Note 6 to the Company's audited consolidated financial statements included elsewhere in this prospectus) included in the Notes required bifurcation and separate accounting as derivatives. The Company bundled these features together and accounted for the features as a single, compound embedded derivative. The Company determined the fair value of the embedded derivative as the difference between the estimated fair value of the respective Notes with and without the redemption features, which resulted in the Company recording the respective Notes at a discount.

The fair value of the bifurcated embedded derivatives as of the respective issuance dates of the 2017 Notes, 2018 Notes, 2019 Notes and 2020 Notes was determined to be $0.3 million, $0.9 million, $0.2 million, and $4.6 million, respectively.

The Company amortized the debt discount over the contractual life of the Notes as a non-cash interest expense utilizing the effective interest method.

As of December 31, 2020, there was no derivative liability due to the conversion of the Notes in June 2020.

Conversion of the Notes

In June 2020, the Company consummated a financing transaction in which it issued shares of Series B redeemable convertible preferred stock. The consummation of this financing transaction resulted in the automatic conversion of the Notes into shares of Series B redeemable convertible preferred stock pursuant to their original terms. Immediately prior to conversion of the Notes, the Company remeasured the fair value of the derivative liability bifurcated from the Notes and recognized changes in the fair value of derivative liability. The Company recognized $1.3 million of net loss related to change in fair value of derivative liability in its condensed consolidated statement of operations for the nine months ended September 30, 2020.

The Company accounted for the conversion of the 2017 Notes and 2018 Notes as a conversion as these Notes converted pursuant to the conversion features in their original terms. Upon the automatic conversion of the 2017 Notes, the Company recorded a contingent beneficial conversion feature of $0.6 million as debt discount that was recognized through interest expense and included in the condensed consolidated statement of operations for the nine months ended September 30, 2020. Additionally, the Company recognized all of the unamortized discount remaining at the date of conversion relating to the original allocation of proceeds to the bifurcated derivative to interest expense. This amounted to $0.1 million and resulted in an increase in the carrying value of the 2017 Notes and an immediate charge to non-cash interest expense, which is included in interest expense in the condensed consolidated statement of operations for the nine months ended September 30, 2020. The Company recorded a total interest expense of $2.3 million in the condensed consolidated statement of operations for the nine months ended September 30, 2020.

Upon conversion of the 2017 Notes and 2018 Notes, the Company derecognized the carrying values of these notes including accrued interest of $16.4 million and recognized Series B redeemable convertible preferred stock.

The Company accounted for the conversion of the 2019 Notes and 2020 Notes as an extinguishment as these notes converted pursuant to redemption features that were bifurcated as embedded derivatives at the original commitment date. The Company recorded a gain on extinguishment of convertible notes of $0.3 million, which is included in other income, net in the condensed consolidated statement of operations for the nine months ended September 30, 2020.

Issuance of the 2021 Notes (the "2021 Notes")

In January 2021, the Company issued, in aggregate, $27.3 million in convertible notes ("2021 Notes") to certain investors, including related parties, of which proceeds of $1.2 million were received in advance of issuance of the 2021 Notes in December 2020 and the remaining proceeds of $26.1 million were received in January and February 2021. The 2021 Notes were to mature on June 30, 2022 and carried both automatic and optional conversion features. The 2021 Notes were secured and carried an interest rate of 3%. The Company recorded the $1.2 million of proceeds received in December 2020 as proceeds received in advance of issuance of 2021 Notes in the condensed consolidated balance sheet as of December 31, 2020, as the subscription agreement and commitment to issue the 2021 Notes was not effective until January 2021.

The 2021 Notes contained the following features:

Automatic Conversion Features—The 2021 Notes were to automatically convert into Conversion Shares upon (i) an IPO, (ii) any transaction in which the Company merges with, consolidates with or enters into other similar transaction with a Special Purpose acquisition Corp ("SPAC"), resulting in some or all of its shares being registered for sale under applicable securities laws and listed for trading on a national or foreign exchange ("De-SPAC transaction"), (iii) the acquisition of the Company by another person or entity by means of any transaction in which holders of the outstanding voting securities of the Company immediately before such transaction held less than 50% of the voting securities of the Company or the surviving corporation after such transaction or a sale of all or substantially all of the assets of the Company but excluding De-SPAC transaction, IPO, and the occurrence of equity financing in which the Company sold shares of its preferred stock for new money and which was neither an IPO or a Qualified Financing ("Change of Control") and (iv) the closing of a sale of an equity transaction in which the Company sold shares with an aggregate gross proceeds of at least $10.0 million ("Qualified Financing").

In the event of a Change of Control, De-SPAC transaction, or an IPO, the Conversion Shares would be common stock of the Company. In the event of a Qualified Financing, the Conversion Shares would be shares of preferred stock issued in such transaction.

Optional Conversion Feature—The holders of the 2021 Notes had the option to elect to convert their notes into Conversion Shares at the Conversion Price upon the occurrence of an equity financing in which the Company sold shares of its preferred stock for new money and which was neither an IPO or a Qualified Financing ("Non-Qualified Financing" and together with the IPO, De-SPAC transaction, Change of Control, and the Qualified Financing, collectively, the "Conversion Events"). In the event of a Non-Qualified Financing, the Conversion Shares would be the class of equity shares issued in such transaction. The 2021 Notes would be deemed to have converted into the Conversion Shares if no election was made by the holders of the 2021 Notes.

Conversion Price—Upon the occurrence of an IPO, the 2021 Notes would convert into shares of common stock at the conversion price equal to the lesser of (i) 85% of the price at which the Company offered each share of common stock in the IPO without deducting any amount for discounts, commissions, fees, or other costs and (ii) $600.0 million divided by the fully diluted capital.

Upon the occurrence of a De-SPAC transaction, the 2021 Notes would convert into shares of common stock at the conversion price equal to the lesser of (i) 85% of the common stock price in the De-SPAC transaction, which would be determined by dividing (x) the total consideration to be paid to common stockholders upon a De-SPAC transaction less the principal amount of the 2021 Notes including accrued and unpaid interest by (y) the common stock issued and outstanding immediately prior to the De-SPAC transaction and that would be exchanged as a result of the De-SPAC transaction including common stock that would be issued upon the exercise of stock options immediately before the Change of Control transaction but excluding the common stock issuable upon conversion of the 2021 Notes and (ii) $600.0 million divided by the fully diluted capital.

Upon the occurrence of a Change of Control, the 2021 Notes would convert into shares of common stock at the conversion price equal to the lesser of (i) 85% of the common stock price in the Change of Control, which would be determined by dividing (x) the total consideration to be paid to common stockholders upon a Change of Control less the principal amount of the 2021 Notes including accrued and unpaid interest by (y) the common stock issued and outstanding immediately prior to the Change of Control and that would be exchanged upon a Change of Control including common stock that would be issued upon the exercise of stock options before the Change of Control but excluding the common stock issuable upon conversion of the 2021 Notes and (ii) $600.0 million divided by the fully diluted capital.

Upon a Qualified Financing, the 2021 Notes would convert into shares of preferred stock issued in the Non-Qualified Financing at the conversion price equal to the lesser of (i) 85% of the lowest price at which the Company sold shares of its stock in the Qualified Financing and (ii) $600.0 million divided by the fully diluted capital.

Repayment—Each holder of the 2021 Notes had the option to elect to receive a payment in the amount equal to the principal amount plus accrued and unpaid interests upon a Change of Control. If a Change of Control occurred and no election was made by the holder, the principal amount and accrued and unpaid interest would be deemed to have automatically be converted into shares of the Company's common stock of the Company immediately prior to the close of the Change of Control.

The Company qualified for and elected to account for the 2021 Notes under the fair value option and, in doing so, bypassed the analysis of potential embedded derivative features. The Company believes that the fair value option better reflects the underlying economics of the 2021 Notes. As a result, the 2021 Notes were recorded at fair value upon issuance, which was determined to be equal to principal

amounts of these notes of $27.3 million. At each financial reporting period, and immediately prior to conversion, the Company remeasured the fair value of the 2021 Notes. The total interest expense and change in fair value of the 2021 Notes from issuance date to the conversion date totaled $5.2 million, which is recorded as change in fair value of convertible notes in the condensed consolidated statement of operations for the nine months ended September 30, 2021. The accrued interest on the 2021 Notes based on the stated interest rate was $0.4 million as of September 30, 2021.

Conversion of the 2021 Notes

In July 2021, the Company consummated a financing transaction in which it issued shares of Series C-1 redeemable convertible preferred stock. The consummation of this financing transaction resulted in the automatic conversion of the 2021 Notes into shares of Series C-2 redeemable convertible preferred stock (together with the Series C-1 redeemable convertible preferred stock, the "Series C Preferred Stock") pursuant to their original terms. The Series C Preferred Stock was determined to have a fair value of $10.265809. Under the fair value option, the 2021 Notes were remeasured to fair value immediately prior to conversion at a price per share equal to the fair value of the Series C-1 redeemable convertible preferred stock. The Company recorded $5.2 million loss related to change in fair value of the 2021 Notes in its condensed consolidated statement of operations for the nine months ended September 30, 2021. The 2021 Notes converted into 3,170,585 shares of Series C-2 redeemable convertible preferred stock at the effective conversion price of $8.725938.

Convertible Notes—Related Parties

There were no convertible notes issued to related parties that were outstanding as of December 31, 2020 and September 30, 2021. In connection with the issuance of the 2021 Notes, the Company issued, in aggregate, $14.3 million of convertible notes to certain related parties. These notes were issued under the same terms and conditions as the 2021 Notes.

7. FAIR VALUE MEASUREMENTS

The following table presents information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values:

	December 31, 2020			
	Level 1	Level 2	Level 3	Total
		(in thousands)		
Assets:				
Money market funds	$10,004	$ —	$ —	$ 10,004
Restricted cash	189	—	—	189
Total financial assets	$10,193	$ —	$ —	$ 10,193

	September 30, 2021			
	Level 1	Level 2	Level 3	Total
		(in thousands)		
Assets:				
Money market funds	$76,273	$ —	$ —	$ 76,273
Short-term investments:				
Commercial paper	—	33,964	—	33,964
Corporate debt securities	—	15,061	—	15,061
Restricted cash	189	—	—	189
Total financial assets	$76,462	$49,025	$ —	$125,487

Valuation of Short-Term Investments

The Company estimates the fair values of the short-term investments by taking into consideration valuations obtained from third-party pricing sources. These pricing sources utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include market pricing based on real-time trade data for the same or similar securities, issuer credit spreads, benchmark yields, and other observable inputs. The Company validates the prices provided by our third-party pricing sources by understanding the models used, obtaining market values from other pricing sources and analyzing pricing data in certain instances.

Valuation of Derivative Liabilities

The fair value of the derivative liabilities was measured using a with-and-without valuation methodology. Inputs used to determine the estimated fair value of the derivative instruments include the probability estimates of potential settlement scenarios for the convertible promissory notes, a present value discount rate and an estimate of the expected timing of settlement. Certain unobservable inputs used in the fair value measurement of the derivative instruments associated with the convertible promissory notes are the scenario probabilities and the discount rate estimated at the valuation date. Generally, an increase or decrease in the discount rate would result in a directionally opposite impact to the fair value measurement of the derivative instruments. Also, a change in the probability scenarios would have varying impacts depending on the weighting of each specific scenario. Heavier weighting toward a qualified financing would result in an increase in the fair value of the derivative liability. Changes in these assumptions can materially affect the fair value.

The following table sets forth the significant inputs to the probability weighted valuation model used to value the derivative liability at issuance of the 2020 Notes:

Type of Event	Expected Date	Probability of Event	Discount Rates	Convertible Notes
Qualified Financing	June 30, 2020	75.0%	30%	2020 Notes
Stock or Asset Sale	March 31, 2021	5.0%	30%	2020 Notes
Note Reaches Maturity	December 31, 2021	20.0%	30%	2020 Notes

The following table sets forth the significant inputs to the probability weighted valuation model used to value the derivative liability upon conversion of the Notes in June 2020:

Type of Event	Expected Date	Probability of Event	Discount Rates	Convertible Notes
Qualified Financing	June 18, 2020	100%	25%	2017, 2018, 2019 and 2020 Notes
Stock or Asset Sale	n/a	0%	25%	2017, 2018, 2019 and 2020 Notes
Note Reaches Maturity	n/a	0%	25%	2017, 2018, 2019 and 2020 Notes

Valuation of the 2021 Notes

At the issuance date of the 2021 Notes, the Company determined that the fair value of the 2021 Notes approximated the principal amounts of the 2021 Notes as the transaction was deemed to be at arm's length. Subsequent measurement of fair value of the 2021 Notes at each reporting period was estimated based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The Company used a scenario-based analysis to incorporate estimates and assumptions concerning the Company's prospects and market indications into a model to estimate the value of the 2021 Notes. The most significant estimates and assumptions

used as inputs are those concerning timing, probability of possible scenarios for conversion or settlement of the 2021 Notes and discount rates. The fair value of the 2021 Notes upon settlement in July 2021 was determined based on the fair value of the Series C-1 redeemable convertible preferred stock issued. This method was selected as the Company concluded that the contemporaneous financing transaction was an arm's length transaction. The issuance of the Series C-1 redeemable convertible preferred stock was considered to be a Qualified Financing (see Note 6) pursuant to the original terms of the 2021 Notes. Accordingly, the fair value calculation for the 2021 Notes immediately before conversion considered both the fair value of the Series C-1 redeemable convertible preferred stock and the conversion price, which was 85% of the fair value of the Series C-1 redeemable convertible preferred stock. The fair value of the 2021 Notes as of June 30, 2021 was determined to be the same as that on the settlement date in July 2021 based on management's determination of no material changes to the assumptions underlying the determination of the fair value of the 2021 Notes.

The following table sets forth the significant inputs to the probability weighted valuation model used to value the 2021 Notes as of March 31, 2021:

Type of Event	Expected Date	Probability of Event	Discount Rates
SPAC / IPO Transaction	August 31, 2021	25%	54.72%
Qualified Financing	June 30, 2021	70%	54.72%
Non-Qualified Financing	June 30, 2021	5%	54.72%
Notes reaches maturity	June 30, 2022	0%	54.72%

The following table represents changes in the derivative liabilities and 2021 Notes with significant unobservable inputs (Level 3):

	Derivative Liability	2021 Notes
	(in thousands)	
Balance as of January 1, 2019	$ 222	$ —
Increase in derivative liability resulting from issuance of convertible notes	4,636	—
Increase in derivative liability resulting from change in estimated fair value	1,270	—
Derivative liability settled upon conversion of convertible notes	(6,128)	—
Balance as of December 31, 2020	$ —	$ —
Initial fair value of convertible notes	—	27,320
Change in fair value of convertible notes	—	5,228
Conversion of convertible notes	—	(32,548)
Balance as of September 30, 2021	$ —	$ —

There were no other assets or liabilities that were measured at fair value on a recurring basis as of December 31, 2020 and September 30, 2021.

8. REDEEMABLE CONVERTIBLE PREFERRED STOCK

In July 2021, the Company consummated a financing transaction in which it issued 13,150,430 shares of Series C-1 redeemable convertible preferred stock. In connection with the issuance of these shares, the principal including accrued interest of the 2021 Notes totaling $27.7 million automatically converted into 3,170,585 shares of Series C-2 redeemable convertible preferred stock.

On July 1, 2021, the Company amended its certificate of incorporation in which it authorized 13,150,430 shares of Series C-1 redeemable convertible preferred stock and 3,170,585 shares of Series C-2 redeemable convertible preferred stock.

The Company's redeemable convertible preferred stock consisted of the following:

	December 31, 2020 (dollars in thousands)				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A preferred stock	6,289,609	6,289,609	$ 7,675	$ 7,730	6,407,259
Series B preferred stock	15,100,000	14,496,835	64,387	246,070	14,496,835
	21,389,609	20,786,444	$ 72,062	$ 253,800	20,904,094

	September 30, 2021 (dollars in thousands)				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A preferred stock	6,289,609	6,289,609	$ 7,675	$ 7,730	6,407,256
Series B preferred stock	15,100,000	14,496,835	64,387	246,070	16,746,059
Series C-1 preferred stock	13,150,430	13,150,430	134,791	135,000	13,150,430
Series C-2 preferred stock	3,170,585	3,170,585	32,498	27,666	3,170,585
	37,710,624	37,107,459	$ 239,351	$ 416,466	39,474,330

As of September 30, 2021, the holders of the Series C Preferred Stock (together with the "Series A redeemable convertible preferred stock" and "Series B redeemable convertible preferred stock", collectively, the "Preferred Stock") have the following rights and preferences:

Conversion – In July 2021, in connection with the conversion of the 2021 Notes, the Company adjusted the conversion price for the Series B redeemable convertible preferred stock of $16.974077 per share to $14.6942. The adjustment was made in accordance with the anti-dilution provisions in the certificate of incorporation then in effect immediately prior to the conversion of the 2021 Notes. The adjustment to the conversion price resulted in additional 2,249,224 shares of common stock into which Series B redeemable convertible preferred stock would be convertible. As of September 30, 2021, these additional shares were not issued and outstanding. Each share of Preferred Stock is convertible into an equivalent number of common stock, at any time, at the option of the holder. The initial conversion price for the Series C-1 redeemable convertible preferred stock and Series C-2 redeemable convertible preferred stock is the respective original issue prices.

The conversion price for the Preferred Stock is subject to adjustments for stock splits, stock dividends, or similar recapitalization, and subject to adjustments in accordance with the anti-dilution provisions.

The shares of Preferred Stock will automatically convert into common stock of the Company immediately upon either (a) the closing of the sale of shares of common stock to the public in a firm commitment underwritten public offering pursuant to an effective registration statement under the Securities Act, resulting in at least $75.0 million of proceeds, net of the underwriting discount and commissions, to the Company (a "Qualified IPO") or (b) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of a majority of the then outstanding shares of Preferred Stock.

Dividends—Dividends may be paid to the holders of the Series A redeemable convertible preferred stock. The holders the Series A redeemable convertible preferred stock are entitled to

receive non-cumulative dividends at a rate per annum of $0.073744 per share when and if declared by the Board of Directors. The holders of the Series B redeemable convertible preferred stock are entitled to receive a non-cumulative dividend at the rate of 6% per annum of the Series B original issue price per share when and if declared by the Board of Directors. As of December 31, 2020, and September 30, 2021, no cash dividends were declared or paid. From and after the date of issuance of the Series C Preferred Stock, the Company will not set, declare, pay or set aside unless holders of the Series C Preferred Stock then outstanding shall first receive, or simultaneously receive, dividends on each outstanding share of Series C Preferred Stock in an amount equal to (i) in the case of dividends being distributed to common stock or any class or series of capital stock that is convertible into common stock, the equivalent dividend on an as-converted basis or (ii) in the case of dividends being distributed on a series or class not convertible into common stock, an additional dividend equal to a dividend rate calculated based on the respective original issue price of the Series C Preferred Stock. The original issue price per share for the Series C-1 redeemable convertible preferred stock and Series C-2 redeemable convertible preferred stock was $10.265809 and $8.725938, respectively.

Voting Rights— The holders of the Preferred Stock are entitled to vote on any matter presented to stockholders of the Company for consideration. Each holder of the Preferred Stock will be entitled to cast the number of votes equal to the number of shares of common stock into which the shares of the Preferred Stock held by such holder are convertible on such date.

Redemption—The Preferred Stock does not contain any mandatory redemption features. In accordance with FASB ASC Topic 480, *Distinguishing Liabilities from Equity (ASC 480)*, preferred stock issued with redemption provisions that are outside of the control of the Company or that contain certain redemption rights in a deemed liquidation event is required to be presented outside of stockholders' deficit on the face of the condensed consolidated balance sheets. The Company classified the Preferred Stock outside of the stockholders' deficit as mezzanine equity because in the event of certain deemed liquidation events, which included events such as a sale or merger, that were not solely within the control of the Company, the shares of the Preferred Stock would become redeemable at the option of the holders. As of December 31, 2020 and September 30, 2021, the Company did not adjust the carrying values of the Preferred Stock to the redemption values of such shares since a deemed liquidation event did not occur and the shares were not probable of becoming redeemable in the future as of the condensed consolidated balance sheet dates.

Liquidation—In the event of a liquidation, deemed liquidation, dissolution or winding up of the Company, holders of the Preferred Stock will be entitled to be paid out of the assets of the Company that are available for distribution before any payment is made to the holders of common stock. The amount to paid will be the greater of (i) the respective original issue prices plus any dividends declared but unpaid or (ii) the amount that would have been payable had all shares of Preferred Stock been converted into common stock immediately before such event. If upon any such liquidation, deemed liquidation, dissolution or winding up of the Company, the assets of the Company available for distribution to its stockholders shall be insufficient to pay the holders of Preferred Stock the full amount to which they shall be entitled, the holders of Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

After the payment of all preferential amounts required to be paid to the holders of Preferred Stock, the remaining assets of the Company available for distribution to its stockholders shall be distributed among the holders of the shares of common stock on a pro rata basis based on the number of shares held by each such holder.

9. STOCKHOLDERS' DEFICIT

Common Stock—The Company had reserved shares of common stock for issuance in connection with the following:

	December 31, 2020	September 30, 2021
Common stock authorized	35,000,000	56,500,000
Common stock issued and outstanding	6,137,206	6,799,157
Common stock authorized and reserved for future issuances:		
Series A redeemable convertible preferred stock	6,407,259	6,407,256
Series B redeemable convertible preferred stock	14,496,835	16,746,059
Series C-1 redeemable convertible preferred stock	—	13,150,430
Series C-2 redeemable convertible preferred stock	—	3,170,585
Common stock reserved for exercise of stock options	3,012,092	4,195,341
Common stock reserved for future issuance of share-based awards	1,170,692	2,809,492
Total common stock authorized and reserved for future issuance	25,086,878	46,479,163
Unreserved common stock available for future issuance	3,775,916	3,221,680

10. STOCK OPTION AND GRANT PLAN

2015 Stock Plan—The Company sponsors the 2015 Plan to encourage and enable the officers, employees, directors, consultants, and other key persons to acquire a proprietary interest in the Company. The 2015 Stock Plan provides for the granting of incentive stock options, non-statutory stock options, and restricted stock awards to eligible employees, officers, directors, consultants, and advisors as determined by the Board of Directors. Terms of restricted stock awards and stock option agreements, including vesting requirements, are determined by the Board of Directors or compensation committee of the Board of Directors, subject to the provisions of the 2015 Plan. The options issued the 2015 Stock Plan expire ten years from the grant date. The options generally vest over four or five years, with 25% vesting on the first anniversary and the balance vesting ratably over the remaining three to four years. Through amendments on December 9, 2015, July 30, 2016, February 15, 2019, February 26, 2020 and July 1, 2021, the total number of share-based awards authorized for issuance was increased to a total of 8,474,374. As of September 30, 2021, there were 2,809,492 common shares available for future grant under the 2015 Plan.

The Company estimates the fair value of stock option awards on the grant date using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Nine Months Ended September 30,	
	2020	2021
Fair value of underlying common stock	$ 2.77	$ 7.12
Risk-free interest rate	1.08%	0.73%
Expected term (in years)	6.05	5.54
Expected volatility	81.64%	76.28%
Dividend yield	0.00%	0.00%

The per share weighted average grant date fair value of stock options granted during the nine months ended September 30, 2020 and 2021 was $1.91 and $4.53, respectively. As of September 30, 2021, total unrecognized compensation expense related to stock options totaled $9.4 million which is expected to be recognized over a weighted average period of 2.4 years.

The following table summarizes the activity under the Company's stock option activity under the 2015 Plan during the nine months ended September 30, 2021:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2020	3,012,092	$ 1.73	6.3	$ 15,501
Granted	1,947,200	$ 7.06		
Exercised	(661,951)	$ 0.30		
Cancelled or forfeited	(102,000)	$ 6.75		
Outstanding as of September 30, 2021	4,195,341	$ 4.31	8.5	$ 17,457
Exercisable as of September 30, 2021	1,320,578	$ 1.13	7.1	$ 9,690
Unvested as of September 30, 2021	2,874,763	$ 5.77	9.1	$ 7,767

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of options exercised during the nine months ended September 30, 2020 and 2021, was $0.1 million and $4.5 million, respectively.

The total fair value of stock options vested during the nine months ended September 30, 2020 and 2021 was $0.1 million and $0.7 million, respectively.

Stock-Based Compensation Expense—The Company recorded stock-based compensation expense in the following expense categories of its condensed consolidated statements of operations:

	Nine Months Ended September 30,	
	2020	2021
	(in thousands)	
Research and development expenses	$ 43	$ 578
General and administrative expenses	79	1,467
Total stock-based compensation	$ 122	$ 2,045

11. NET LOSS PER SHARE

Net Loss per Share Attributable to Common Stockholders—Because the Company reports a net loss attributable to common stockholders, basic and diluted net loss per share attributable to common stockholders are the same for both periods presented. All preferred stock and stock options have been excluded from the computation of diluted weighted-average shares outstanding because such securities would have an antidilutive impact. The following common stock equivalents outstanding at each period end have been excluded from the calculation of diluted net loss per share because their inclusion would have been antidilutive:

	Nine Months Ended September 31,	
	2020	2021
Options to purchase common stock	2,633,870	4,195,341
Redeemable convertible preferred stock	20,904,094	39,474,330

12. RELATED PARTY TRANSACTIONS

Convertible Notes

In connection with the issuance of the 2021 Notes, the Company issued, in aggregate, $14.3 million of convertible promissory notes to Morningside Ventures Investments Limited, and certain members of the board of directors of the Company. Morningside Ventures Investments Limited is a 5% significant stockholder and has appointed representatives to the board of directors of the Company. Morningside Ventures Investments Limited has designated a member to the board of directors of the Company. These notes were issued under the same terms and conditions as the 2021 Notes (see Note 6).

13. COMMITMENTS AND CONTINGENCIES

Operating Leases—The Company's lease commitments did not materially change during the nine months ended September 30, 2021.

Letter of Credit—Restricted cash consists of cash serving as collateral for a letter of credit issued for the Company's office space. As of December 31, 2020 and September 31, 2021, the Company's restricted cash balance was $0.2 million on its condensed consolidated balance sheets.

Legal Proceedings—The Company does not believe that it is party to any pending legal proceedings that are likely to have a material effect on its business, financial condition, or results of operations for the nine months ended September 30, 2021. At each reporting date, the Company evaluates whether or not a potential loss amount or potential range of loss is probable and reasonably estimated under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company recognizes expenses for its costs related to its legal proceedings, as incurred.

Royalty Payments—The Company's commitments under the royalty agreements with the Grantors did not materially change during the nine months ended September 30, 2021. The Company did not record a liability for future royalty payments in the condensed consolidated balance sheets as of December 31, 2020 and September 30, 2021 as the achievement and timing of the future royalty payments were not probable or estimable.

14. SUBSEQUENT EVENTS

For its condensed consolidated financial statements, the Company has performed an evaluation of subsequent events through November 24, 2021, which is the date the condensed consolidated financial statements were issued.

PPP Loan

In October 2021, the Company repaid the $0.3 million outstanding balance of its PPP Loan. See Note 5, *Accrued Expenses*, to these notes to condensed consolidated financial statements for additional information regarding the loan.

Shares

Common Stock

Prospectus

Goldman Sachs & Co. LLC **SVB Leerink** **Evercore ISI**

H.C. Wainwright & Co.

, 2022

Through and including , 2022, (the 25th day after the date of this prospectus), all dealers effecting transactions in the common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

PART II

INFORMATION NOT REQUIRED IN PROSPECTUS

Item 13. Other Expenses of Issuance and Distribution.

The following table sets forth the expenses to be incurred in connection with the offering described in this Registration Statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimates except the Securities and Exchange Commission's registration fee, the Financial Industry Regulatory Authority, Inc. filing fee and the exchange listing fee.

	Amount
Securities and Exchange Commission registration fee	$ *
Financial Industry Regulatory Authority, Inc. filing fee	*
Exchange listing fee	*
Accountants' fees and expenses	*
Legal fees and expenses	*
Transfer Agent's fees and expenses	*
Printing and engraving expenses	*
Miscellaneous fees and expenses	*
Total expenses	$ *

* To be filed by amendment.

Item 14. Indemnification of Directors and Officers.

Section 145 of the Delaware General Corporation Law, or the DGCL, authorizes a corporation to indemnify its directors and officers against liabilities arising out of actions, suits and proceedings to which they are made or threatened to be made a party by reason of the fact that they have served or are currently serving as a director or officer to a corporation. The indemnity may cover expenses (including attorneys' fees) judgments, fines and amounts paid in settlement actually and reasonably incurred by the director or officer in connection with any such action, suit or proceeding if the director or officer acted in good faith and in a manner the director or officer reasonably believed to be in or not opposed to the best interests of the corporation, and, with respect to any criminal action or proceeding, had no reasonable cause to believe the director or officer's conduct was unlawful. Section 145 permits corporations to pay expenses (including attorneys' fees) incurred by directors and officers in advance of the final disposition of such action, suit or proceeding upon receipt of an undertaking by or on behalf of such director or officer to repay such amount if it shall ultimately be determined that such director or officer is not entitled to be indemnified by the corporation as authorized in Section 145. In addition, Section 145 provides that a corporation has the power to purchase and maintain insurance on behalf of its directors and officers against any liability asserted against them and incurred by them in their capacity as a director or officer, or arising out of their status as such, whether or not the corporation would have the power to indemnify the director or officer against such liability under Section 145.

We have adopted provisions in both our certificate of incorporation and bylaws to be in effect upon the completion of this offering that limit or eliminate the personal liability of our directors to the fullest extent permitted by the DGCL, as it now exists or may in the future be amended. Consequently, a director will not be personally liable to us or our stockholders for monetary damages or breach of fiduciary duty as a director, except for liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;

- any unlawful payments related to dividends or unlawful stock purchases, redemptions or other distributions; or
- any transaction from which the director derived an improper personal benefit.

These limitations of liability do not alter director liability under the federal securities laws and do not affect the availability of equitable remedies such as an injunction or rescission.

In addition, our bylaws provide that:

- we will indemnify our directors, officers and, in the discretion of our board of directors, certain employees to the fullest extent permitted by the DGCL, as it now exists or may in the future be amended; and
- we will advance reasonable expenses, including attorneys' fees, to our directors and, in the discretion of our board of directors, to our officers and certain employees, in connection with legal proceedings relating to their service for or on behalf of us, subject to limited exceptions.

We have entered into indemnification agreements with each of our directors and intend to enter into such agreements with certain of our executive officers. These agreements provide that we will indemnify each of our directors, certain of our executive officers and, at times, their affiliates to the fullest extent permitted by Delaware law. We will advance expenses, including attorneys' fees (but excluding judgments, fines and settlement amounts), to each indemnified director, executive officer or affiliate in connection with any proceeding in which indemnification is available and we will indemnify our directors and officers for any action or proceeding arising out of that person's services as a director or officer brought on behalf of us or in furtherance of our rights. Additionally, certain of our directors or officers may have certain rights to indemnification, advancement of expenses or insurance provided by their affiliates or other third parties, which indemnification relates to and might apply to the same proceedings arising out of such director's or officer's services as a director referenced herein. Nonetheless, we have agreed in the indemnification agreements that our obligations to those same directors or officers are primary and any obligation of such affiliates or other third parties to advance expenses or to provide indemnification for the expenses or liabilities incurred by those directors are secondary.

We also maintain general liability insurance which covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers, including liabilities under the Securities Act.

The underwriting agreement filed as Exhibit 1.1 to this registration statement provides for indemnification of us and our directors and officers by the underwriters against certain liabilities under the Securities Act and the Exchange Act.

Item 15. Recent Sales of Unregistered Securities.

Set forth below is information regarding shares of our common stock and shares of our preferred stock issued, and stock options granted, by us within the past three years that were not registered under the Securities Act. Included is the consideration, if any, we received for such shares and options and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, under which exemption from registration was claimed.

(a) Issuances of Convertible Notes

On November 9, 2018, we issued convertible promissory notes to various investors in the principal amount of $12,978,100.00.

From December 17, 2019 to April 22, 2020, we issued convertible promissory notes to various investors in the principal amount of $16,042,503.77.

From January 27, 2021 to February 9, 2021, we issued convertible promissory notes to various investors in the principal amount of $27,320,508.40.

No underwriters were involved in the foregoing issuances of securities. The securities described in this section (a) of Item 15 were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(a)(2) under the Securities Act and Regulation D promulgated thereunder relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. The recipients of securities in the transactions described above represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

(b) Issuances of Capital Stock.

In June 2020, we issued and sold an aggregate of 14,496,835 shares of Series B preferred stock at a price per share of $16.974077, for an aggregate purchase price of approximately $64.4 million. Included in this amount was approximately $34.4 million of outstanding principal and interest on the convertible promissory notes issued between July 2017 and April 2020, all of which converted into Series B preferred stock in this financing in accordance with their terms. In July 2021, we issued and sold an aggregate of 13,150,430 shares of Series C-1 preferred stock at a price per share of $10.265809 and 3,170,585 shares of Series C-2 preferred stock at a price per share of $8.725938, for an aggregate purchase price of approximately $162.7 million. Included in this amount was approximately $27.7 million of outstanding principal and interest on the convertible promissory notes issued between January 2021 and February 2021, all of which converted into Series C-2 preferred stock in this financing in accordance with their terms.

No underwriters were involved in the foregoing issuance of securities. The securities described in this section (b) of Item 15 were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(a)(2) under the Securities Act and Regulation D promulgated thereunder relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. The recipients of securities in the transactions described above represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

(c) Stock Option Grants and Option Exercises.

As of the date of this registration statement, we have granted options to purchase an aggregate of 5,766,882 shares of common stock, with exercise prices ranging from $0.001 to $7.57 per share, to employees, directors, and consultants pursuant to our 2015 Plan. Of these, options for 119,000 shares have been terminated, options for 1,662,067 shares have been exercised, and options for 3,985,815 shares remain outstanding. 2,826,492 shares remain available for grant under our 2015 Plan.

No underwriters were involved in the foregoing issuances of securities. The issuances of stock options described in this paragraph (c) of Item 15 were issued pursuant to written compensatory plans or arrangements with our employees, directors, consultants and advisors, in reliance on the exemption

provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(a)(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

Item 16. Exhibits and Financial Statement Schedules.

(a) Exhibits.

The exhibits to the registration statement are listed in the Exhibit Index attached hereto and incorporated by reference herein.

(b) Financial Statement Schedules.

No financial statement schedules are provided because the information called for is not required or is shown either in the financial statements or the related notes.

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
1.1*	Form of Underwriting Agreement.
3.1**	Third Amended and Restated Certificate of Incorporation of the Registrant (as currently in effect).
3.2**	Amended and Restated Bylaws of the Registrant (as currently in effect).
3.3*	Form of Restated Certificate of Incorporation of the Registrant (to be effective prior to the closing of this offering).
3.4*	Form of Amended and Restated Bylaws of the Registrant (to be effective prior to the closing of this offering).
4.1*	Specimen stock certificate evidencing the shares of common stock.
4.2**	Second Amended and Restated Investors' Rights Agreement, dated as of July 1, 2021, among the Registrant and the other parties thereto.
5.1*	Opinion of Goodwin Procter LLP.
10.1#*	2015 Stock Option and Incentive Plan and forms of award agreements thereunder.
10.2#*	2022 Stock Option and Incentive Plan and form of award agreements thereunder.
10.3#*	2022 Employee Stock Purchase Plan.
10.4#*	Senior Executive Cash Incentive Bonus Plan.
10.5#*	Non-Employee Director Compensation Plan.
10.6*	Lease Agreement, dated as of October 23, 2018, as amended, by and between the Registrant and Bullfinch Square Limited Partnership.
10.7#*	Employment Agreement, between the Registrant and Josh Cohen (to be entered into in connection with this offering).
10.8#*	Employment Agreement, between the Registrant and Justin Klee (to be entered into in connection with this offering).
10.9#*	Employment Agreement, between the Registrant and James Frates (to be entered into in connection with this offering).
10.10#*	Employment Agreement, between the Registrant and Margaret Olinger (to be entered into in connection with this offering).
10.11#*	Employment Agreement, between the Registrant and Patrick D. Yeramian, M.D. (to be entered into in connection with this offering).
10.12#*	Form of Director Indemnification Agreement.
10.13#*	Form of Officer Indemnification Agreement.
10.14+	Master Manufacturing Services Agreement, dated as of November 12, 2019, by and between the Registrant and Patheon Inc.
10.15+	Supply Agreement, dated as of October 29, 2019, by and between the Registrant and CU Chemie Uetikon GmbH.
10.16+	Research, Development and Supply Agreement, dated as of December 9, 2019, and Deed of Amendment, dated as of July 26, 2021, by and between the Registrant and ICE S.p.A. (formerly Prodotti Chimici e Alimentari S.p.A.), as amended.

Exhibit Number	Description of Exhibit
21.1	Subsidiaries of the Registrant.
23.1*	Consent of Deloitte & Touche LLP, independent registered public accounting firm.
23.2*	Consent of Goodwin Procter LLP (included in Exhibit 5.1).
24.1*	Power of Attorney (included on signature page).

* To be filed by amendment.
** Previously filed.
\+ Portions of this exhibit (indicated by asterisks) have been omitted in accordance with Item 601(b)(10) of Regulation S-K.
\# Indicates a management contract or any compensatory plan, contract or arrangement.

Item 17. Undertakings.

(a) The undersigned registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

(b) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

(c) The undersigned registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this Registration Statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Cambridge, Commonwealth of Massachusetts, on this day of , 2022.

AMYLYX PHARMACEUTICALS, INC.

By: _____
Joshua Cohen
Co-Chief Executive Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Joshua Cohen and Justin Klee and each of them, his true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution for him and in his name, place and stead, in any and all capacities, to sign any and all amendments (including post-effective amendments) to this registration statement, and any subsequent registration statements pursuant to Rule 462 of the Securities Act and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that each of said attorney-in-fact or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
Joshua Cohen	Co-Chief Executive Officer and Director (Principal Executive Officer)	, 2022
Justin Klee	Co-Chief Executive Officer and Director (Principal Executive Officer)	, 2022
James M. Frates	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	, 2022
George Mclean Milne Jr, Ph.D.	Director	, 2022
Paul Fonteyne	Director	, 2022
Felix Von Coerper	Director	, 2022
Isaac Cheng, M.D.	Director	, 2022
Daphne Quimi	Director	, 2022

Exhibit 10.14

Certain identified information has been excluded from this exhibit because it is both not material and is the type that the registrant treats as private or confidential. Information that was omitted has been noted in this document with a placeholder identified by the mark "[***]".

Master Manufacturing Services Agreement

Effective Date: November 12, 2019

PARTIES

PATHEON INC.
a company existing under the laws of Canada, with a place of business at 111 Consumers Drive, Whitby, Ontario L1N 5Z5 ("**Patheon**"),

- and -

AMYLYX PHARMACEUTICALS, INC.
a corporation existing under the laws of the State of Delaware, with its principal place of business located at 43 Thorndike Street, Cambridge, MA 02141 ("**Client**").

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1. **Structure of Agreement and Interpretation**

1.1 Master Agreement.

This Master Manufacturing Services Agreement (the "Agreement") establishes the general terms and conditions under which Patheon or any Affiliate of Patheon in the business of performing manufacturing services may perform Manufacturing Services for Client or any Affiliate of Client. This master form of agreement is intended to allow the parties, or any of their Affiliates, to contract for the manufacture of Product through Patheon's global network of manufacturing sites by entering into specific Product Agreements without having to re-negotiate the general terms and conditions that apply.

1.2 Product Agreements.

This Agreement is structured so that Product Agreements may be entered into by the parties (or their Affiliates) for the manufacture of Product at any Patheon manufacturing site. Each Product Agreement will be governed by and will incorporate the terms and conditions of this Agreement, except to the extent that the parties to the Product Agreement expressly modify the terms and conditions of this Agreement in the Product Agreement. Unless otherwise agreed by the parties, each Product Agreement will be substantially in the general form, and contain the information referred to, in Appendix 1. In the event of any conflict between the terms of this Agreement and a particular Product Agreement, the terms and conditions of the Product Agreement shall control if so specifically stated; otherwise, the terms and conditions of this Agreement shall control for all purposes.

1.3 Definitions.

The following terms will, unless the context otherwise requires, have the respective meanings set out below and grammatical variations of these terms will have corresponding meanings:

"**Affiliate**" means:

(a) a business entity which owns, directly or indirectly, a controlling interest in a party; or

(b) a business entity which is controlled by a party, either directly or indirectly; or

(c) a business entity, the controlling interest of which is directly or indirectly common to the majority ownership of a party;

For this definition, "control" means the lawful right to determine (by ownership of shares or otherwise) the election of the majority of directors (or equivalent managers) of a business entity;

"**Annual Volume**" means, for the purpose of the Price, Patheon's assumed volume of Product to be manufactured in any Year as set out in the "Annual Volume Forecast" section of Schedule A of the applicable Product Agreement.

"**API**" means the active materials listed in the applicable Product Agreement (references to "Active Materials" or "Active Pharmaceutical Ingredient" in documents forming part of this Agreement or of a Product Agreement will mean "API");

"**API Credit Value**" means the value of the API for certain purposes of this Agreement, as set out in the applicable Product Agreement;

"**Applicable Laws**" means: (i) for Patheon, the Laws of the jurisdiction where the Manufacturing Site is located; and (ii) for Client and the Product, the Laws of all jurisdictions where Product is manufactured, distributed, and marketed as these are agreed by the parties in the Product Agreement;

"**Authority**" means any governmental or regulatory authority, department, body or agency or any court, tribunal, bureau, commission or other similar body, whether federal, state, provincial, county or municipal, with competent jurisdiction over a party, the Manufacturing Services, or the relevant Product (or its use);

"**Batch**" means a quantity of Product in kilograms or units as provided under the applicable Product Agreement.

"**Business Day**" means a day other than a Saturday, Sunday or a day that is a statutory holiday in Ontario, Canada;

"**Capital Equipment Agreement**" means the separate agreement that the parties may enter into that addresses the rights and responsibilities of the parties regarding capital equipment and facility modifications that may be required to perform the Manufacturing Services under a particular Product Agreement;

"**cGMPs**" means, as applicable, current good manufacturing practices as described in:

(a) Parts 210 and 211 of Title 21 of the United States' Code of Federal Regulations;

(b) Commission Directive (EU) 2017/1572 (art. 2); and

(c) Division 2 of Part C of the Food and Drug Regulations (Canada); together with current final industry-accepted Health Canada, FDA and EMA guidance documents pertaining to manufacturing and quality control practice, all as updated, amended and revised from time to time;

"**Client Intellectual Property**" means: Intellectual Property provided to Patheon by or on behalf of Client, or generated or derived by Client before the Effective Date of this Agreement or by Patheon while performing any Manufacturing Services which Intellectual Property is specific to, or dependent upon, the Product;

"**Client-Supplied Components**" means those Components supplied or to be supplied by or on behalf of Client as identified in Schedule A of a Product Agreement;

"**Components**" means, collectively, all packaging components, raw materials, ingredients, and other materials (including labels, product inserts and other labelling for the Products) required to manufacture or package Product in accordance with the Processing Instructions, other than the API;

"**Confidential Information**" has the meaning specified in Section 11.1;

"**Conversion Fee**" means the capital price for performing the Manufacturing Services excluding the cost of Components as specified in the Product Agreement;

"**DEA**" means the Drug Enforcement Administration of the United States Department of Justice;

"**Deficient Product**" has the meaning specified in Section 6.1(a);

"**Disclosing Party**" has the meaning specified in Section 11.1;

"**EMA**" means the European Medicines Agency;

"**FDA**" means the United States Food and Drug Administration;

"**Firm Order**" has the meaning specified in Section 5.1(d);

"**Health Canada**" means the department of the Canadian Government known as Health Canada and includes, among other relevant branches, the Therapeutic Products Directorate and the Health Products and Food Branch Inspectorate;

"**Initial Product Term**" has the meaning specified in Section 8.1;

"**Intellectual Property**" includes, without limitation, rights in patents, patent applications, formulae, trademarks, trademark applications, trade-names, Inventions, copyrights, industrial designs, confidential information, trade secrets, materials, data, writings, and know how;

"**Invention**" means information about any innovation, improvement, development, discovery, computer program, device, trade secret, method, process, technique or the like, whether or not written or otherwise fixed in any form or medium, regardless of the media on which it is contained and whether or not patentable or copyrightable;

"**Inventory**" means, at a point in time, all inventories of Components and work-in-process under Patheon's care or control used for the manufacture or packaging of Product;

"**Launch Period**" means the [***] period commencing on the date of the first commercial sale of a Product with all Regulatory Approvals to the general public in the Territory and ending on the [***] anniversary of the first commercial sale. For example, if the first commercial sale occurred on August 1,2020 then the Launch Period would terminate on [***].

"**Laws**" means all laws, statutes, ordinances, regulations, rules, by-laws, judgments, decrees or orders of any Authority;

"**Local Currency**" has the meaning specified in Appendix 4;

"**Long Term Forecast**" has the meaning specified in Section 5.1(a);

"**Manufacturing Services**" means the manufacturing, quality control, quality assurance, stability testing, packaging, and related services, as set out in this Agreement or in any Product Agreement, for the manufacture of Product for distribution in the Territory;

"**Manufacturing Site**" means the facility identified in a Product Agreement where the Manufacturing Services will be performed;

"**Minimum Market Requirement**" has the meaning specified in Section 2.1;

"**Minimum Order Quantity**" means, for each manufacturing campaign ordered, the minimum number of units, batches or kilograms of a Product that Client must purchase, as set out in Schedule A of the applicable Product Agreement;

"**Obsolete Stock**" has the meaning specified in Section 5.2(b);

"**Patheon Competitor**" means a business that derives greater than fifty (50%) percent of its revenues from performing contract pharmaceutical or biopharmaceutical development or commercial manufacturing services;

"**Patheon Intellectual Property**" means Intellectual Property generated or derived by Patheon or its Affiliates before performing any Manufacturing Services, developed by Patheon while performing the Manufacturing Services, or otherwise generated or derived by Patheon in its business which Intellectual Property is not specific to or dependent upon the Product or Client's confidential information related to the Product including, without limitation, Inventions and Intellectual Property which apply to manufacturing processes or the formulation or development of drug products or drug delivery systems unrelated to the specific requirements of the Product and specifically excluding all Client Intellectual Property and Client Confidential Information;

"**Partial Assignment**" means the asset sale by Client of a Product.

"**Price**" means the fees to be charged by Patheon for:

(a) performing the Manufacturing Services;

(b) the cost of Components (other than Client-Supplied Components); and

(c) any separate cost items and other fees,

as set out in Schedule A of the applicable Product Agreement;

"**Processing Instructions**" means the agreed file, for each Product, which contains documents relating to the Product, including, without limitation:

(a) quality control testing methods for API and Components;

(b) manufacturing instructions, directions, and processes;

(c) any storage requirements for the API, Components, or Product;

(d) all environmental, health and safety information for the Product including material safety data sheets; and

(e) the finished Product quality control testing methods, packaging instructions and shipping requirements for the Product;

"**Product**" means a product listed in Schedule A of a Product Agreement;

"**Product Agreement**" means the agreement between Patheon and Client (or their applicable Affiliates) substantially in the form set out in Appendix 1 under which Patheon will perform Manufacturing Services;

"**Product Claims**" has the meaning specified in Section 6.1(a);

"**Quality Agreement**" means a separate agreement that sets out the quality assurance standards for the Manufacturing Services;

"**Recall**" has the meaning specified in Section 6.2(a);

"**Recipient**" has the meaning specified in Section 11.1;

"**Regulatory Approval**" has the meaning specified in Section 7.5(a);

"**Regulatory Authority**" means the FDA, EMA, and Health Canada and any other foreign regulatory agencies competent to grant marketing approvals for pharmaceutical or biopharmaceutical products, including the Products, in the Territory;

"**Release Date**" means in relation to each batch of Product the scheduled date by which the Product will be released by Patheon's quality department (by confirmation or certification) as agreed in the Quality Agreement and made available for shipment, and as confirmed by Patheon in a Firm Order;

"**Representatives**" means, a party's directors, officers, employees, advisers, agents, consultants, subcontractors, service partners or professional advisors;

"**Rolling Forecast**" has the meaning specified in Section 5.1(b);

"**Technical Dispute**" has the meaning specified in Appendix 2;

"**Territory**" means the geographic area described in a Product Agreement where Product manufactured by Patheon will be distributed by or on behalf of Client;

"**Third Party Rights**" means the Intellectual Property of any third party;

"**VAT**" has the meaning specified in Section 13.14; and

"**Year**" means in the first year of this Agreement or a Product Agreement, the time from the Effective Date up to and including December 31 of the same calendar year, and after that will mean a calendar year.

1.4 Interpretation.

The division of this Agreement into Sections, Subsections, Appendices and Schedules, and the insertion of headings, are for convenience of reference only and will not affect the interpretation of this Agreement. Unless otherwise indicated, any reference in this Agreement to a Section, Appendix or Schedule refers to the specified Section, Appendix or Schedule to this Agreement. In this Agreement, the term "**this Agreement**" and similar expressions refer to this Agreement as a whole and not to any particular part, Section, Appendix or Schedule of this Agreement. Except as otherwise expressly stated or unless the context otherwise requires, all references to the singular will include the plural and vice versa.

2. **Patheon's Manufacturing Services**

2.1 Manufacturing Services.

In accordance with the terms of this Agreement, Patheon will timely perform the Manufacturing Services as set out in the relevant Product Agreement for the Price and in accordance with the Quality Agreement. Subject to the preceding sentence, Patheon will convert API and Components into Product, and provide all supportive and related Manufacturing Services such as quality assurance (for example quality controls, analytical testing, and stability programs), primary and secondary packaging, and any other related Manufacturing Services as agreed to between the parties. During the Launch Period, Patheon will manufacture One Hundred (100%) percent of Client's Product offered for sale by Client or its Affiliates (the "**Initial Market Requirement**"). Following the Launch Period, Client agrees that it will order from Patheon the percentage of its manufacturing requirements in the Territory as set forth in the negotiated Product Agreement (the "**Minimum Market Requirement**").

2.2 Subcontracting.

Patheon may, with the prior written consent of the Client, subcontract the Manufacturing Services under a Product Agreement to any of its Affiliates, as agreed in the Product Agreement. Patheon will remain exclusively liable to Client for any breach of this Agreement or negligence by its Affiliates in the course of performing: (i) subcontracted Manufacturing Services under a Product Agreement; or (ii) obligations under the Quality Agreement. Patheon may also arrange for non-Affiliate subcontractors to perform specific services arising under any Product Agreement with the prior written consent of Client ("**Third Party Subcontractors**"). Patheon will be liable to Client for the breach of this Agreement or negligence of the Third Party Subcontractor or the failure by any Third Party Subcontractor to perform any part of the subcontracted services. But Patheon's liability for Third Party Subcontractors will remain subject to all limitations on Patheon's liability as set out in this Agreement. Patheon will have no liability arising from the performance of services by Third Party Subcontractors: (i) that are chosen by Client; (ii) that are suppliers or service providers not validated and utilized by Patheon prior to the date of this Agreement; (iii) that are supplying materials or supplies required to perform the services; or (iv) to the extent that the Third Party Subcontractor is following the direct instructions of Client.

3. **Client's Obligations**

3.1 Payment.

Subject to Appendix 2 of this Agreement, Client will pay Patheon the applicable Price in accordance with Sections 4 and 5. All cost items that are not included in the Price (as specified in the applicable Product Agreement) are subject to additional fees to be paid by Client.

3.2 Processing Instructions.

Before the start of commercial manufacturing of Product under this Agreement, Client will give Patheon a copy of the Processing Instructions or, together with Patheon, develop and implement such instructions, which must be accompanied by the applicable API, Component and finished product specifications (if applicable, precisely matching the specifications approved by the applicable Regulatory Authority). If the Processing Instructions or accompanying documents received are amended or no longer reflect those currently approved by the Regulatory Authority, then Client will give Patheon a copy of the revised documents (if applicable, precisely matching the revised specifications approved by the applicable Regulatory Authority). Upon acceptance of the revised Processing Instructions and accompanying documents, Patheon will give Client a signed and dated receipt indicating Patheon's acceptance. At Patheon's request, Client will provide evidence of the executed original documents submitted by or on behalf of Client to the Regulatory Authority.

3.3 API and Components.

(a) Client will, at its sole cost and expense, deliver the API and any Client-Supplied Components to the Manufacturing Site DDP (Incoterms 2010). Client's obligation with respect to such delivery will consist of obtaining the release of the API and any Client-Supplied Components from the applicable customs agency and Regulatory Authority. Unless otherwise agreed in writing, Client or Client's designated broker will be the "**Importer**" or "**Importer of Record**" (or equivalent, as understood under Applicable Laws) for API, Client-Supplied Components, drug products and intermediates imported to the Manufacturing Site, and Client is responsible for compliance with Applicable Laws (and the cost of compliance) relating to that role. For API or Client-Supplied Components which may be subject to import or export to or from the United States, Client agrees that its vendors and carriers will comply with applicable requirements of the U.S. Customs and Border Protection Service and the Customs Trade Partnership Against Terrorism.

(b) Unless otherwise agreed in writing between the parties, the API and any Client-Supplied Components must be delivered by the Client to the Manufacturing Site at least [***] before the scheduled manufacture date for Product covered by a Firm Order in sufficient quantity to enable Patheon to manufacture the agreed quantities of Product. Patheon reserves the right to refuse to store any quantity of API in excess of the amount necessary for the Firm Order, at its sole discretion at any time but will use its commercially reasonable efforts to store such excess quantities. If Client fails to deliver the API or Client-Supplied Components within the agreed time period and, after making commercially reasonable efforts, Patheon is unable to manufacture Product on the scheduled date because of the delay, the Firm Order will be considered cancelled by Client and Section 5.1(e) will apply.

(c) Patheon will control the unloading of API and Client-Supplied Components arriving at the Manufacturing Site and Client will comply and ensure that its carrier complies with all related directions of Patheon. The API and Client-Supplied Components will be held by Patheon on behalf of Client as set out in this Agreement. The API and Client-Supplied Components will at all times remain the property of Client. Any API and Client-Supplied Components received by Patheon will only be used by Patheon to perform the Manufacturing Services.

(d) Client will ensure that: (i) all delivered API meets the specifications for that API and (ii) all shipments of API are accompanied by the required documentation as specified in the applicable Quality Agreement.

(e) If Client asks Patheon to qualify an additional supplier for the API or any Component, the parties must agree on the scope of work to be performed by Patheon and the additional fees to be paid by Client. For any API or any Component, this work at a minimum will include: (i) laboratory testing to confirm the API or Component meets existing specifications; (ii) manufacture of an experimental batch of Product that will be placed on [***] accelerated stability; and (iii) manufacture of full-scale validation batches that will be placed on concurrent stability (one batch may be the registration batch if manufactured at full scale).

Patheon will promptly advise Client if it encounters API or Client-Supplied Component or Component supply problems, including delays or delivery of non-conforming API or Components from a Client designated additional supplier. The parties will cooperate to reduce or eliminate any supply problems from these additional suppliers. If supply problems persist, Patheon may suspend the Manufacturing Services affected by the problems until it is satisfied that the Client has resolved the problems with its supplier or appointed an alternative supplier. Client will qualify or certify (as appropriate) all Client designated additional suppliers on an annual basis at its expense and will provide Patheon with copies of the relevant annual reports. If Patheon agrees to certify or qualify a Client designated additional supplier on behalf of Client, it will do so for an additional agreed-upon fee payable by Client.

3.4 Packaging and Artwork.

Client will be responsible for the cost of artwork development and approval of all artwork. Client will be responsible for changes to labels, product inserts, and other packaging for the Product, including obtaining all required approvals. Client will be responsible for the cost of labelling obsolescence as contemplated in Section 5.2. Patheon's name will not appear on the label or anywhere else on the Product unless: (i) required by any Laws; or (ii) Patheon consents in writing to the use of its name. At least [***] prior to the Release Date of Product for which new or modified artwork is required, Client will provide at no cost to Patheon and in accordance with the applicable specifications, final camera ready artwork for all packaging Components to be used in the manufacture of the Product. Client will be responsible for the costs associated with complying with any and all regulatory requirements for the labelling and tracking of the manufactured Product, including product serialisation, product data transfer and anticounterfeiting requirements in the Territory.

4. Price and Price Adjustments

4.1 First Year Pricing.

The Price for each Product will be listed in Schedule A of a Product Agreement and may be adjusted under this Section 4.

4.2 Annual Price Adjustments.

Patheon may adjust the Price effective [***] as follows:

(a) Inflation. Patheon may adjust the Price for inflation in accordance with Appendix 4.

(b) Currency Fluctuations. If the parties agree in a Product Agreement to invoice in a currency other than the Local Currency for the Manufacturing Site, Patheon will adjust the Price to reflect currency fluctuations. The adjustment will be calculated in accordance with Appendix 4 after all other annual Price adjustments under this Section 4.2 have been made.

(c) Pricing Basis. Client and Patheon acknowledge and agree that the Price in any Year is to be mutually negotiated and agreed upon. The factors to consider include the applicable Minimum Market Requirement, Annual Volume, and Minimum Order Quantity for that Year. Patheon may adjust the Price if it reasonably concludes, or is notified by Client, that the Minimum Market Requirement, Annual Volume or Minimum Order Quantity will not be ordered in a Year.

(d) Tier Pricing. If the Pricing is divided into Annual Volume tiers, unless otherwise agreed in a Product Agreement, Client will be invoiced during the Year based at the lowest volume tier. Within [***] after the end of each Year and on termination of the Product Agreement, Patheon will send Client a reconciliation of the actual volume of Product ordered by Client during the Year at the actual applicable Pricing tiers. If the reconciliation shows an overpayment, Patheon will issue a credit to Client for the amount for the amount of the overpayment within [***] after the end of the Year or will reimburse the overpayment within [***] after the end of such Year or of such termination. The parties will use their commercially reasonable efforts and work together in good faith to resolve any disagreement over the reconciliation.

For all Price adjustments under this Section 4.2, Patheon will deliver to Client on or about [***] (unless otherwise agreed in writing) a letter stating the adjusted Pricing under a Product Agreement to be effective for Product to be delivered on or after [***] including any Firm Orders accepted by Patheon before that date.

4.3 Price Adjustments at any Time.

The Prices may be adjusted by Patheon at any time upon written notice to Client as follows:

(a) Extraordinary Increases in Component Costs. If the cost of a Component increases cumulatively by at least [***] percent since the last annual adjustment as a result of market factors outside of Patheon's control, then Patheon will be entitled to adjust the Price proportionately and as otherwise agreed in the Product Agreement. The revised Price will become effective with [***]. For a Price adjustment under this Section 4.3(a), Patheon will deliver to Client a revised Schedule A to the Product Agreement.

(b) Changes. The scope of the Manufacturing Services is set by the agreed Processing Instructions, the Regulatory Approvals, the Quality Agreement and any assumptions, inclusions, exclusions and other parameters set out in the applicable Product Agreement. Changes to the scope of the Manufacturing Services and related changes to the Price must be agreed in writing by the parties (using a "**Change of Scope**" agreement, or similar, setting out the agreed activities and costs of implementation) and are subject to the change control provisions of the Quality Agreement. Where Patheon requests a change to the Manufacturing Services, the change will be implemented following written approval of Client, which Client will not unreasonably withhold, condition or delay.

5. **Purchasing Product**

5.1 Orders and Forecasts.

(a) Long Term Forecast. On or before June 1 of each Year, Client will give Patheon a non-binding written forecast of Client's volume requirements for the Product for each of the next [***] ("**Long Term Forecast**"). If Patheon foresees any capacity constraint affecting any portion of the Long Term Forecast, it will notify Client within [***] following receipt of the Long Term Forecast, and the parties will agree on a revised Long Term Forecast within Patheon's expected capacity. Each updated Long Term Forecast supersedes all previous Long Term Forecasts. All forecasts under this Agreement shall specify the number of Batches or units, as applicable.

(b) Rolling Forecast. Before each Product Agreement is executed, Client will give Patheon a written forecast of the volume of Product that Client expects to order in each of the next [***] (the "**Rolling Forecast**"). Client will use commercially reasonable efforts to make the Rolling Forecast consistent with the most recent Long Term Forecast. Client will provide an updated Rolling Forecast: (i) on or before the [***] of each month; and (ii) if at any time it determines that the total forecast volumes estimated in the most recent Rolling Forecast have changed by more than [***] percent. Each updated Rolling Forecast supersedes all previous Rolling Forecasts.

(c) Orders. On or before the [***] of each month, Client will issue a new purchase order for any required Product. Each purchase order must meet the Minimum Order Quantity as noted in the applicable Product Agreement and specify the purchase order number, quantities by Product type, and requested release dates for the Product (which must occur at least [***] after the first day of the next month).

(d) Acceptance of Purchase Orders. To the extent that a purchase order covers Product that is forecast in the Rolling Forecast, Patheon will accept the purchase order by sending an acknowledgement to Client, including the confirmed Release Dates. Subject to Section 5.1(f), if Patheon fails to acknowledge receipt of a purchase order within [***], the purchase order will be considered accepted by Patheon. An accepted purchase order will be binding on the parties (a "**Firm Order**"), except that either party may request to change any Release Date beyond [***] after the first day of the next month. The parties will negotiate in good faith and agree on any requested alternative release date. Neither party may unreasonably reject an alternative release date requested under this Section 5.1(d), but, if the parties cannot agree, the original Release Date confirmed by Patheon will apply.

(e) Cancellation or Postponement. Patheon will determine the manufacturing schedule of all Product covered by Firm Orders which shall be consistent with Client's delivery requirements. If Client cancels or reduces a Firm Order, or wishes to postpone the applicable Release Date (subject to Section 5.1(d)), Client will remain liable to pay Patheon [***] percent of the Price for the Firm Order.

(f) Capacity Reservation. On July 1st in the last Year of the Launch Period, Patheon will use the Rolling Forecast to reserve its manufacturing capacity in the next Year for Product.

In all other cases, by reference to the Rolling Forecast applicable at June 1 of each Year, the relevant forecast for the Year being the "**Yearly Forecast Volume**".

At the end of each Year after the Launch Period, if the aggregate actual Batches ordered by Client with a confirmed Release Date within the Year, taking into account any Product paid for but not ordered, ("**Actual Yearly Volume**") is less than [***] of the Yearly Forecast Volume, then Patheon may invoice and Client will pay Patheon the Conversion Fee portion of the Price for the shortfall of Product below the tolerance during the Year in an amount calculated as follows:

Amount Due to Patheon
= *((Yearly Forecast Volume x* [***]) — *Actual Yearly Volume')* x *Conversion Price for the Product*

If the quantity of Product requested by Client in a Year (in purchase orders received by Patheon) exceeds the Yearly Forecast Volume for that Year, Patheon will use commercially reasonable efforts to supply the additional Product volumes. Patheon will not be considered to have accepted any purchase order for additional Product volumes without written confirmation.

(g) Controlled Substance Quota Requirements (if applicable). Client will give Patheon the information set out below for obtaining any required DEA or equivalent agency quotas ("**Quota**") needed to perform the Manufacturing Services. Patheon will be responsible for routine management of Quota information in accordance with Applicable Laws. The parties will cooperate to communicate the information and to assist each other in Regulatory Authority information requirements related to the Product as follows: (i) by April 1 of each Year for the applicable Product, Client will provide to Patheon the next

Year's annual Quota requirements for the Product; (ii) by [***], Client will provide to Patheon any changes to the next Year's Quota requirements; (iii) Client will pro-actively communicate any changes to the Quota requirements for the then-current Year in sufficient time to allow Patheon to file and finalize Regulatory Authority filings supporting the changes; (iv) upon Patheon receiving the necessary forecast information from Client in order to request additional Quota, Patheon will submit to the applicable Regulatory Authority, on a timely basis, all filings necessary to obtain Quotas for API and will use commercially reasonable efforts to secure sufficient Quota from the applicable Regulatory Authority so as to achieve Release Dates for Product as set out in applicable purchase orders and forecasts submitted to Patheon by Client or its designee; and (v) Patheon will not be responsible for any Regulatory Authority's refusal or failure to grant sufficient Quota for reasons beyond the reasonable control of Patheon (including where Client fails to provide the required information in accordance with this Section 5.1(g)).

5.2 Obsolete Stock.

(a) Client understands and acknowledges that Patheon will rely on purchase orders, Firm Orders, the Long Term Forecast and the Rolling Forecast in ordering the Components (other than Client-Supplied Components) required to meet anticipated Firm Orders. Patheon may purchase the Components in sufficient volumes, and reasonably in advance of the expected use of the Component (taking into account lead times), to meet the production requirements for Products covered by anticipated Firm Orders or for the Rolling Forecast to meet the production requirements of any other amounts agreed to by the parties.

(b) For Components ordered by Patheon in relation to Firm Orders or under Section (a) that are not used in the Manufacturing Services within [***] after the forecasted month for which the purchases have been made due to actual orders being less than the Firm Orders or Rolling Forecasts, or if the Components have expired or are rendered obsolete due to Client- required changes in any Processing Instructions, GMP, or artwork, or due to changes in Applicable Laws during the period (collectively, "**Obsolete Stock**"). Client shall either (i) at Client's cost (plus a [***] handling fee) pick up from Patheon's Manufacturing Site all obsolete stock or (ii) reimburse Patheon for the cost of obsolete stock which reimbursement will include Patheon's cost to purchase and destroy the Obsolete Stock (plus a [***] percent handling fee). Client shall elect either (i) or (ii) within [***] of receipt of written notice from Patheon identifying the Obsolete Stock. If any non-expired Components are used in Products subsequently manufactured for Client or in third party products manufactured by Patheon, Client will receive credit for any costs of those Components previously paid to Patheon by Client.

5.3 Storage.

If: (i) Client fails to take possession or arrange for the destruction of Obsolete Stock within [***] of receipt of written notice from Patheon identifying the Obsolete Stock; (ii) any equipment (other than existing Patheon equipment) is stored at the Manufacturing Site at any time prior to its use in the Manufacturing Services; or (iii) Product is not collected by Client within [***] of the Release Date notified by Patheon, Client will pay Patheon USD [***] per pallet, per month after that for storing the Obsolete Stock, equipment or Product. Storage fees for Obsolete Stock or Product which contain controlled substances or require refrigeration will be charged at USD [***] per pallet per month. Storage fees are subject to a one (1) pallet minimum charge per month. Patheon may ship Product held by it longer than [***] to Client at Client's expense on [***] written notice to Client. If Patheon is unable to store any material due to capacity constraints, Patheon may use an Affiliate or qualified third party to store (outside the Manufacturing Site) any material under this Agreement. After the limited storage periods stated above, Client will assume all risk of loss or damage to materials and Client will be responsible for having appropriate insurance coverage in place for this risk.

5.4 Invoices and Payment.

For shipments of Product, Patheon will issue invoices to Client on or after the Release Date of the Product. Otherwise, Patheon will issue invoices for Manufacturing Services on completion or as agreed in the Product Agreement. Patheon will also submit to Client, with each shipment of Product, a duplicate copy of the invoice covering the shipment. Invoices will be sent by email to the email address given by Client to Patheon in writing. Each invoice will, to the extent applicable, identify Client's Manufacturing Services purchase order number, Product numbers, names and quantities, unit price, freight charges, and the total amount to be paid by Client. Client will pay all undisputed invoices within [***] of the date of the invoice. If any portion of an invoice is disputed, Client will pay Patheon for the undisputed amount and the parties will use good faith efforts to reconcile the disputed amount as soon as practicable. Interest on undisputed past due accounts will accrue at [***] per month. Patheon may, on giving [***] notice to Client, suspend all Manufacturing Services, including release and shipment of Product, until all undisputed past due invoices have been paid in full. Patheon will have no liability to Client for losses caused by this suspension, including without limitation, losses due to delayed Product delivery or Product shortages.

5.5 Delivery and Shipping.

Delivery of Product and any other materials will be made EXW (Incoterms 2010) from Patheon's Manufacturing Site unless otherwise agreed in a Product Agreement. Subject to Section 8.3, risk of loss or of damage to Product will remain with Patheon until Patheon loads the Product onto the carrier's vehicle for shipment at the shipping point at which time risk of loss or damage will transfer to Client. But if Client fails to collect Product within [***] after it has been released for shipment by Patheon, Client will assume all risk of loss or damage to the released Product. Patheon may, in accordance with Client's instructions and as agent for Client, at Client's risk, arrange for shipping (to Client or any third party nominated by Client) to be paid by Client. Client will arrange for insurance and will select the freight carrier used by Patheon to ship Product and may monitor Patheon's shipping and freight activity under this Agreement.

6. **Product Claims and Recalls**

6.1 Product Claims.

(a) Rejection. Client may reject any manufactured Product that it reasonably considers (by reference to the results of the agreed release testing) to be deficient based on: (i) documentation provided by Patheon, (ii) Client's own inspection, (iii) testing of delivered Product or (iv) is not manufactured in accordance with the Processing Instructions, Product Agreement or this Agreement.

(b) Product Claims.

(i) Client may claim a remedy (a "**Product Claim**") for any portion of any batch of Product for which Patheon did not perform the Manufacturing Services in accordance with the agreed Processing Instructions, cGMPs, or Applicable Laws ("**Deficient Product**"). Client will inspect Product manufactured by Patheon, or batch documentation provided by Patheon, upon receipt and will give Patheon written notice of all Product Claims within [***] after receipt (or, in the case of any deficiency not susceptible to discovery upon receipt, within [***] after discovery by Client, but not after the expiration date of the Product). If Client fails to provide a Product Claim within the applicable [***] period, then the Product will be considered to have been accepted by Client on the [***]. Patheon will have no liability for any deficiency for which it has not received notice within the applicable [***] period.

(ii) This Section 6 sets out the only liability of Patheon for Deficient Products. Patheon will provide a remedy for Product Claims as specified in Section 10.2, but Patheon will have no obligation for any Product Claims to the extent the Deficient Product was caused by: (i) deficiencies in the Processing Instructions, specifications, the safety, efficacy, or marketability of the Product or its distribution; (ii) a defect in the API or an incorporated Component that was not reasonably discoverable by Patheon using the test methods set out in the Processing Instructions; (iii) actions of Client or third parties occurring after the Product is delivered by Patheon; (iv) packaging design or labelling defects or omissions for which Patheon has no responsibility; (v) any unascertainable reason despite Patheon having performed the Manufacturing Services in accordance with the Processing Instructions, cGMPs, and Applicable Laws; or (v) any other breach by Client of its obligations under this Agreement that resulted in a Deficient Product. If after a full investigation as set out in the Quality Agreement and this Section 6.1 (b)(ii), it is determined that Patheon manufactured Product in accordance with the agreed Processing Instructions, but a batch or portion of batch of Product is not released, Client will pay Patheon the Price for the Product. Patheon's only liability for API loss is set out in Appendix 3.

(c) Determination of Deficiency. Upon receipt of a Product Claim, Patheon will have [***] to advise Client by notice in writing if it disagrees with the contents of the Product Claim. If the parties fail to agree within [***] after Patheon's notice to Client as to whether any Product identified in the Product Claim is Deficient Product, the parties will investigate the matter in accordance with the Quality Agreement. If, after joint testing or investigation has been performed, the parties still cannot agree on the root cause, the provisions of Appendix 2 will apply and, after the required negotiation, the dispute will be handled as a Technical Dispute.

(d) Shortages and Price Disputes. Claims for shortages in the amount of Product shipped by Patheon or a Price dispute will be dealt with by reasonable agreement of the parties. Any claim for a shortage or a Price dispute will be considered waived by Client if it has not been presented within [***] of the date of the relevant invoice.

6.2 Product Recalls and Returns.

(a) Records and Notice. The parties will each maintain records necessary to permit a Recall of any Product delivered to Client or customers of Client. Each party will promptly notify the other of any information which might affect the marketability, safety or effectiveness of the Product or which might result in the Recall or seizure of the Product in accordance with the Quality Agreement. Upon receiving this notice or upon this discovery, each party will stop making any further shipments of any Product in its possession or control until a decision has been made whether a Recall or some other corrective action is necessary. The decision to initiate a Recall or to take some other corrective action, if any, will be made and implemented by Client. "**Recall**" will mean any action: (i) by Client to recover title to or possession of quantities of the Product sold or shipped to third parties (including, without limitation, the voluntary withdrawal of Product from the market); (ii) by any Regulatory Authority to detain or destroy any of the Product; or (iii) by either party to refrain from selling or shipping quantities of the Product to third parties which would be subject to a Recall if sold or shipped.

(b) Recalls. If: (i) any Regulatory Authority issues a directive, order or, following the issuance of a safety warning or alert about a Product, a written request that any Product be Recalled; (ii) a court of competent jurisdiction orders a Recall; or (iii) Client determines that any Product should be Recalled or that a "**Dear Doctor**" letter is required relating the restrictions on the use of any Product, then Patheon will co-operate as reasonably required by Client, having regard to all Applicable Laws.

(c) Recalled Product. To the extent that a Recall results from, or arises from Deficient Product, Patheon will be responsible for all reasonable documented out-of-pocket expenses of the Recall and will replace the Deficient Product with replacement Products as per Section 10. In all other circumstances, Recalls, returns, or other corrective actions will be made at Client's cost and expense unless caused by the negligent action or inaction of Patheon. Patheon's only liability for API loss is set out in Appendix 3.

6.3 Disposition of Deficient Product.

Client will not dispose of any damaged, returned, or Deficient Product for which it intends to assert a Product Claim against Patheon without Patheon's prior written authorization to do so. Patheon may instruct Client to return the Products to Patheon. Patheon will bear the cost of return and disposition of any Deficient Products. In all other circumstances, Client will bear the cost of return and disposition, including all applicable fees for Manufacturing Services.

7. Co-operation and Regulatory Affairs

7.1 Governance.

Each party will without delay upon execution of this Agreement or a Product Agreement appoint one of its employees to be a relationship manager responsible for liaison between the parties. The relationship managers will meet on a frequency agreed to between the parties to review the current status of the business relationship, including review of key performance indicators such as API delivery, on-time delivery, right first time, attainment of the Minimum Market Requirement and manage any issues that have arisen.

7.2 Governmental Agencies.

Subject to any restrictions in the Quality Agreement, either party may communicate with any Regulatory Authority responsible for granting Regulatory Approval for the Product and any other relevant Authority regarding the Product if, in the opinion of that party's counsel, the communication is necessary to comply with the terms of this Agreement or the requirements of the Authority or Applicable Laws. Otherwise, the parties will consult each other in relation to regulatory communications relating to the Product in accordance with the Quality Agreement.

7.3 Records.

Patheon will keep records of the manufacture, testing, and shipping of the Product, and retain samples of the Product as are necessary to comply with all manufacturing regulatory requirements applicable to Patheon, Applicable Laws, cGMP and the Quality Agreement. Copies of the records and samples will be retained as and for the period specified in the Quality Agreement or longer as may be required by any Applicable Law. Patheon reserves the right to destroy or return to Client, at Client's sole expense, any document or samples for which the retention period has expired if Client fails to arrange for destruction or return within [***] of receipt of written notice from Patheon.

7.4 Audits.

Subject to the limits agreed in the Quality Agreement, Patheon will give Client access at mutually agreed times to the areas of the Manufacturing Site in which the Product is manufactured, stored, handled, or shipped to permit Client to verify that the Manufacturing Services are being performed in accordance with the specifications, Product Agreement, cGMPs, and Applicable Laws. If Client wishes to audit Patheon beyond the agreed limits, except where the audit is required due to Patheon's breach, Client will pay to Patheon a fee of USD [***] for each additional audit day and USD [***] per audit day for each additional auditor. Under no circumstances will: (a) Client have a right of access to Patheon's financial record; or (b) any Patheon Competitor be permitted access to the Manufacturing Site.

7.5 Regulatory Filings.

(a) Regulatory Authority Documentation. Client will provide copies of all relevant documents relating to Regulatory Authority approval for the commercial manufacture, distribution and sale of the Product ("**Regulatory Approval**") to Patheon on request and as required under the Quality Agreement. Patheon will review and verify the accuracy of these documents in accordance with the Quality Agreement. Client is not entitled to submit Regulatory Approvals referring to Patheon or its Affiliates or the Services until approved by Patheon.

(b) Deficiencies. If, in Patheon's sole discretion, acting reasonably, Patheon determines that any regulatory information given by Client is inaccurate or deficient in any manner whatsoever (the "**Deficiencies**"), Patheon will notify Client in writing of the Deficiencies. The parties will work together to have the Deficiencies resolved prior to the date of filing of the relevant application and in any event before any pre-approval inspection or before the Product is placed on the market if a pre-approval inspection is not performed.

(c) Inspection by Regulatory Authorities. If Client does not give Patheon the documents requested under this Section 7.5 or the Quality Agreement within the time specified and if Patheon reasonably believes that Patheon's standing with a Regulatory Authority may be jeopardized, Patheon may, in its sole discretion, delay or postpone any inspection by the Regulatory Authority until Patheon has reviewed the requested documents and is satisfied with their contents. Client's breach of this requirement will be considered a material breach of this Agreement.

(d) Pharmacovigilance. Client will be responsible, at its expense, for all pharmacovigilance obligations for the Product in accordance with Applicable Laws and the monitoring and management of post-marketing complaints and queries at its cost (including, without limitation, the cost of assistance required of Patheon under the Quality Agreement). Unless required by Applicable Law, neither party will be obliged to exchange with the other party any information or data which it compiles in carrying out pharmacovigilance obligations or activities.

(e) No Patheon Responsibility. Except as otherwise agreed in the Quality Agreement, Patheon will not assume any responsibility for: (a) the submission, accuracy or cost of any application for Regulatory Approval or related documentation (or the success of those applications); (b) any activity that is required by Applicable Laws for Regulatory Approval (including pharmacovigilance and complaints handling, and preparation and submission of any regular quality or other update); or (c) any dealings with the relevant Regulatory Authority on behalf of Client for Regulatory Approval. If a Regulatory Authority, or other governmental body, requires Patheon to incur fees, costs or activities in relation to the Products which Patheon considers unexpected and extraordinary, then Patheon will notify Client in writing and the parties will discuss in good faith appropriate mutually acceptable actions, including fee/cost sharing, or termination of all or any part of this Agreement or a Product Agreement. Patheon will be not be obliged to undertake these activities or to pay for the fees or costs until the parties reach agreement on scope and fees for Patheon's assistance.

7.6 Release.

The parties agree that the release of the Products for sale or distribution under the applicable marketing approval for the Product will not by itself indicate compliance by Patheon with its obligations relating to the Manufacturing Services or the applicable Product Agreement. Nothing in this Agreement will remove or limit the authority of the relevant quality function (as specified by the Quality Agreement) to determine whether the Product will be released for sale or distribution.

7.7 Withdrawal on Completion.

No later than [***] following completion or permanent cessation of the Manufacturing Services at the applicable Manufacturing Site, Client will: (a) ensure that any regulatory filings relating to the Product are withdrawn or amended to remove all references to the Manufacturing Site and, as applicable, Patheon or its Affiliates and their facilities (except in an historic context); and (b) provide to Patheon written confirmation of its compliance with this Section 7.7. If this time is not sufficient to meet the requirements of certain Regulatory Authorities, despite Client's best efforts, then Patheon shall agree to reasonably extend the period based on the written reassurances of Client.

8. Term and Termination

8.1 Initial Term.

This Agreement will become effective as of the Effective Date and will continue until December 31 2025 (the "**Initial Term**"), unless terminated earlier by one of the parties. This Agreement will automatically renew after the Initial Term if there is a Product Agreement in effect and will terminate co-terminus with the last Product Agreement to expire unless otherwise agreed by the Parties. In any event, the legal terms and conditions of this Agreement will continue to govern any Product Agreement in effect. Each Product Agreement will have an initial term from the Effective Date of the Product Agreement until the date agreed to by the Parties (each, an "**Initial Product Term**").

8.2 Termination for Cause.

(a) Either party may terminate this Agreement or a Product Agreement upon written notice where the other party has failed to remedy a material breach of this Agreement or the Product Agreement within [***] (the "**Remediation Period**") following receipt of a written notice of the breach from the aggrieved party that expressly states that it is a notice under this Section 8.2(a) (a "**Breach Notice**"). In the event of any conflict, the terms of this Agreement will supercede the terms and conditions of the Product Agreement unless

otherwise specifically agreed to in writing. The aggrieved party's right to terminate this Agreement or a Product Agreement under this Section 8.2(a) may only be exercised for a period of [***] following the expiry of the Remediation Period (where the breach has not been remedied) and if the termination right is not exercised during this period then the aggrieved party will be considered to have waived the breach described in the Breach Notice and if timely exercised this Agreement or a Product Agreement shall accordingly be terminated and be declared null and void except for those provisions which survive in accordance with their terms. The right to terminate a Product Agreement under this Section 8.2(a) does not extend to any other Product Agreements where there has been no material breach of those other Product Agreements. Notwithstanding anything to the contrary herein, in the event Patheon is not able to timely manufacture and deliver the Product in accordance with Section 2.1 due to Patheon's negligence or failure to perform under the terms hereof then the Client shall have the right , upon written notice to Patheon, to secure alternative manufacturing services. Client's rights in this Section 8.2(a) shall continue so long as Patheon is unable to comply with the terms of Section 2.1 due to its negligence or failure to perform under this Agreement.

(b) Either party may immediately terminate this Agreement or a Product Agreement upon written notice to the other party if: (i) the other party is declared insolvent or bankrupt by a court of competent jurisdiction; (ii) a voluntary petition of bankruptcy or insolvency is filed in any court of competent jurisdiction by the other party; or (iii) this Agreement or a Product Agreement is assigned by the other party for the benefit of creditors.

(c) Client may terminate a Product Agreement upon [***] prior written notice if any Authority takes any action, or raises any objection, that permanently prevents Client from selling the Product in the Territory.

(d) Client may terminate a Product Agreement upon [***] prior written notice if it intends to no longer order Manufacturing Services for a Product due to the Product's discontinuance in the market.

(e) Patheon may terminate this Agreement or any Product Agreement upon [***] prior written notice if Client assigns under Section 13.4 any of its rights under this Agreement or a Product Agreement to an assignee that, in the reasonable opinion of Patheon, is: (i) unlikely to be able to meet the obligations of this Agreement or a Product Agreement; (ii) is as of the date of the assignment a Patheon Competitor as supported by reasonable evidence; or (iii) an entity with whom Patheon has had prior unsatisfactory business relations (as supported by reasonable evidence of late or unpaid invoices or material disputes).

(f) Patheon may terminate this Agreement or any Product Agreement if payment in full of overdue, undisputed invoices is not received within [***] following the date of suspension of Manufacturing Services by Patheon under Section 5.4.

(g) If Client forecasts zero volume for [***] during the term of a Product Agreement (excluding regulatory issues or the registration period), then Patheon may terminate the Product Agreement by providing [***] prior written notice to Client. Within that [***] period, Client may either: (i) withdraw the zero forecast and re-submit a reasonable volume forecast, after which Patheon will withdraw the termination notice or (ii) negotiate other terms and conditions on which the Product Agreement will remain in effect.

8.3 Obligations on Termination.

If a Product Agreement is completed, expires, or is terminated in whole or in part for any reason, then:

(a) Client will take delivery of and pay for all undelivered Products that are manufactured or packaged in accordance with this Agreement under a Firm Order, at the Price in effect at the time the Firm Order was released;

(b) Client will purchase all Inventory that was purchased (or will be purchased under existing unfulfilled orders for Components), maintained or produced by Patheon in contemplation of filling Firm Orders or in accordance with Section 5.2, at Patheon's cost (including all costs incurred by Patheon for the purchase, handling, and processing of the Inventory);

(c) Client, at its own expense, will remove from the Manufacturing Site, within [***] following the completion, termination, or expiration of the Product Agreement, all unused API and Client-Supplied Components, all applicable Inventory (whether current or obsolete), supplies, undelivered Product, chattels, equipment or other moveable property owned by Client, related to the Agreement and located at the Manufacturing Site or that is otherwise under Patheon's care and control ("**Client Property**"). If Client fails to remove Client Property within the [***] period, which may be extended by the Client for unforeseen circumstances, for up to an additional [***] period, Client will then pay Patheon [***] per pallet, per month, one pallet minimum (except that Client will pay [***] per pallet, per month, one pallet minimum, for any of Client Property that contains controlled substances, requires refrigeration or other special storage requirements) after that for storing Client Property and will assume any third party storage charges invoiced to Patheon regarding Client Property (which Patheon may incur at its discretion). Patheon may ship Client Property to Client or to an external warehouse at Client's risk and expense. Patheon will invoice Client for these storage charges as set out in Section 5.3 of this Agreement. If Client fails to remove Client Property within [***] following the completion, termination, or expiration of the Product Agreement, which may be extended by the Client for unforeseen circumstances, for up to an additional [***] period, Client will assume all risk of loss or damage to the stored Client Property and it will be Client's responsibility to have appropriate insurance coverage in place for this risk. If Client asks Patheon to destroy any Client Property, Client will be responsible for the cost of destruction; and

(d) any completion, termination or expiration of this Agreement or a Product Agreement will not affect any prior outstanding obligations or undisputed payments due nor will it prejudice any other remedies that the parties may have under this Agreement or a Product Agreement or any related Capital Equipment Agreement. Completion, termination or expiration of this Agreement or of a Product Agreement for any reason will not affect the obligations and responsibilities of the parties under Sections 5.1(e), 5.1(f), 5.4, 5.5, 8.3, 10, 11, 12, 13.14, 13.15 and 13.16, all of which shall survive any completion, termination or expiration of this Agreement of a Product Agreement, as well as any other provisions that are by implication or otherwise intended to survive any completion, termination or expiration. Where Patheon has agreed to provide stability services beyond the final supply of Product, the relevant provisions of this Agreement will survive for the agreed duration of those stability services.

8.4 Technology Transfer.

Following termination of a Product Agreement for any reason, or at Client's request within [***] before the end of the term of a Product Agreement, Patheon will provide assistance to transfer all of Client's manufacturing process, know-how and analytical testing methodology for the Product to Client ("Technology Transfer") to assist Client to manufacture the Product. Patheon will also disclose to Client any Patheon Intellectual Property that is required or helpful to manufacture the Product. Patheon will, upon request of Client, prepare a written proposal to perform the Technology Transfer. Client will pay the mutually agreed reasonable fee for the Technology Transfer performed by Patheon.

9. **Representations, Warranties and Covenants**

9.1 Authority.

Each party covenants, represents, and warrants that it has the full right and authority to enter into this Agreement and inter-related agreements and that it is not aware of any impediment that would inhibit its ability to perform its obligations under this Agreement or any inter-related agreements.

9.2 Client Warranties.

(a) Non-lnfrinqement. Client covenants, represents, and warrants that:

(i) the Processing Instructions and specifications for the Product are its or its Affiliate's property and that Client may lawfully disclose the Processing Instructions and specifications to Patheon for use in accordance with this Agreement;

(ii) any Client Intellectual Property used by Patheon in performing the Manufacturing Services: (A) is Client's or its Affiliate's unencumbered property, (B) may be lawfully used as directed by Client and agreed in this Agreement and (C) to the Client's knowledge, does not infringe any Third Party Rights;

(iii) the performance of the Manufacturing Services by Patheon or the use or other disposition of any Product by Patheon as may be required to perform its obligations under this Agreement or any Product Agreement does not, to the Client's knowledge, infringe any Third Party Rights; and (iv) to the Client's knowledge, there are no actions or other legal proceedings involving Client or its Affiliates that concerns the infringement of Third Party Rights related to any of the Processing Instructions or specifications, or any of the API or Client-Supplied Components, or the sale, use, or other disposition of Product made in accordance with the Processing Instructions.

(b) Quality and Compliance. Client covenants, represents, and warrants that:

(i) the Processing Instructions and specifications for the Product conforms to all applicable cGMPs and Applicable Laws;

(ii) the Product, if labelled and manufactured in accordance with the Processing Instructions and in compliance with applicable cGMPs and Applicable Laws: (i) may be lawfully sold and distributed in every jurisdiction in which Client markets the Product, (ii) will be fit for the purpose intended and (iii) will be safe for human consumption; and (iii) on receipt by Patheon, the API will conform to the specifications for the API that Client has given to Patheon and that the API will be adequately contained, packaged, and labelled in accordance with Applicable Laws and will conform to the affirmations of fact on the container.

9.3 Patheon Warranties.

Patheon covenants, represents, and warrants that:

(a) it will perform the Manufacturing Services with experienced, trained employees and qualified subcontractors and in accordance with this Agreement, a Quality Agreement, a Product Agreement, the Processing Instructions, cGMPs, and Applicable Laws;

(b) any Patheon Intellectual Property used by Patheon to perform the Manufacturing Services: (i) is Patheon's or its Affiliate's unencumbered property, (ii) may be lawfully used by Patheon, and (iii) to Patheon's knowledge, does not infringe and will not infringe any Third Party Rights;

(c) it will not in the performance of its obligations under this Agreement use the services of any person it knows is debarred or suspended under 21 U.S.C. §335(a) or (b); and

(d) it does not currently have, and it will not hire, as an officer or an employee any person whom it knows has been convicted of a felony under the laws of the United States for conduct relating to the regulation of any drug product under the United States Federal Food, Drug, and Cosmetic Act.

(e) Patheon shall perform all of its obligations under this Agreement in full compliance with all Applicable Laws in the Territory. Patheon shall hold during the Term of this Agreement all licenses, permits and similar authorizations required by any Regulatory Authority in the Territory for Patheon to perform its obligations under this Agreement.

(f) The Product furnished by Patheon to Client under this Agreement:

(i) will perform the Manufacturing Services in accordance with the agreed Processing Instructions, cGMPs, or Applicable Laws and shall be manufactured, packaged, labelled, handled, stored and shipped in compliance with all applicable Laws including the agreed Processing Instructions, cGMPs, and in accordance with the Quality Agreement; and

(ii) shall only contain Product material that has been used, handled, or stored in accordance with the specifications, all applicable Laws and the Quality Agreements;

9.4 Permits.

(a) Client will be solely responsible for obtaining or maintaining, on a timely basis, any permits or other regulatory approvals for the Product, Processing Instructions or specifications, including, without limitation, all marketing and post-marketing approvals, and any specific approvals referred to in the Quality Agreement.

(b) Patheon will maintain at all relevant times when performing the Manufacturing Services during the Term of this Agreement, all required governmental permits, licenses, approval, and authorities.

9.5 No Warranty.

PATHEON MAKES NO WARRANTY OR CONDITION OF ANY KIND, EITHER EXPRESSED OR IMPLIED, BY FACT OR LAW, OTHER THAN THOSE EXPRESSLY SET OUT IN THIS AGREEMENT. PATHEON MAKES NO WARRANTY OR CONDITION OF FITNESS FOR A PARTICULAR PURPOSE NOR ANY WARRANTY OR CONDITION OF MERCHANTABILITY FOR THE PRODUCT.

10. **Liability and Remedies**

10.1 Consequential and Other Damages.

Under no circumstances whatsoever will either party be liable to the other in contract, tort, negligence, indemnity, breach of statutory duty, or otherwise for: (i) any (direct or indirect) delay, penalty, loss of profits, of anticipated savings, of business, of goodwill, or of use of the Product or costs of any substitute services; or (ii) any reliance damages, including but not limited to costs or expenditures incurred to evaluate the viability of entering into this Agreement or to prepare for performance under this Agreement; or (iii) for any other liability, damage, costs, penalty, or expense of any kind incurred by the other party of an indirect or consequential nature, regardless of any notice of the possibility of these damages.

10.2 Limitation of Liability.

(a) Remedies for Deficient Product. If Client makes a Product Claim under Section 6.1 and the parties agree the Product is a Deficient Product, or the Product is determined to be a Deficient Product under Section 6, Patheon will promptly, at Client's election, either:

(i) replace the Product at Patheon's cost (after which Patheon may invoice for the replacement) if Patheon is able to manufacture the replacement Product at the Manufacturing Site and contingent upon the receipt from Client of all API and Client- Supplied Components required for the manufacture of the replacement Product; or

(ii) refund [***] percent of the Price paid for the Deficient Product (by credit or offset against other amounts due to Patheon under the Product Agreement).

Except for the indemnity set out in Section 10.3 and any claim for expenses related to a Recall under Section 6.2(c), the remedies described in this Section 10.2 will be Client's sole remedy in contract, tort, negligence, equity or otherwise, for Deficient Product.

The remedy under this Section 10.2, if applicable (including in the case of Recall), will apply only to the extent that the affected Deficient Product is unsold and returned, destroyed or otherwise disposed of by Client in accordance with this Agreement.

(b) API. Except as expressly set out in Appendix 3, under no circumstances whatsoever will Patheon be liable to Client in contract, tort, negligence, indemnity, breach of statutory duty, or otherwise for any loss or damage to the API. Patheon's maximum aggregate liability for loss of or damage to the API will not exceed on a per Product basis [***] percent of revenues (being payments of the Price) received by Patheon for that Product under the applicable Product Agreement during the previous Year (or, in the case of the first Year, the expected revenue for that Product if the agreed Yearly Forecast Volumes were ordered).

(c) Maximum Liability. In any Year, in addition to the specific remedies under Section 10.2(a) for Deficient Product, Patheon's maximum aggregate liability to Client under or in connection with this Agreement or any Product Agreement (however arising, including contract, tort, negligence, indemnity, breach of statutory duty, losses of API, or otherwise) will not exceed on a per Product basis [***] percent of revenues (being payments of the Price) received by Patheon for that Product under the applicable Product Agreement during the previous Year (or, in the case of the first Year, the expected revenue for that Product if the agreed Yearly Forecast Volumes were ordered).

(d) Death, Personal Injury and Fraudulent Misrepresentation. Nothing contained in this Agreement will act to exclude or limit either party's liability for personal injury or death caused by the negligence of either party or fraudulent misrepresentation.

10.3 Patheon Indemnity.

(a) Patheon agrees to defend, indemnify and hold harmless Client, its officers and employees, against all losses, damages, costs, claims, demands, subpoenas, judgments and liability to, from and in favour of third parties (other than Affiliates) for i) any claim of infringement of any Third Party Rights by Patheon Intellectual Property, ii) for any claim of a breach of any warranty or representation by Patheon or iii) for any claim of personal injury or property damage to the extent that the injury or damage is the result of a failure by Patheon to perform the Manufacturing Services in accordance with the Processing Instructions, cGMPs, and Applicable Laws except to the extent that the losses, damages, costs, claims, demands, subpoenas, judgments, and liability are due to the negligence or wrongful acts of Client, its officers, employees, or Affiliates.

(b) If a claim occurs, Client will: (i) promptly notify Patheon of the claim; (ii) use commercially reasonable efforts to mitigate the effects of the claim; (iii) reasonably cooperate with Patheon in the defense of the claim; and (iv) permit Patheon to control the defense and settlement of the claim, all at Patheon's cost and expense; provided that Client consents in writing to any settlement.

10.4 Client Indemnity.

(a) Client agrees to defend and indemnify and hold harmless Patheon, its officers and employees, against all losses, damages, costs, claims, demands, subpoenas, judgments and liability to, from and in favour of third parties (other than Affiliates) for i) any claim of infringement of any Third Party Rights in or the Products or that relates to the manufacture of the Product by a proprietary process disclosed by Client or to Patheon's use of Client's Intellectual Property to perform the Manufacturing Services, or any portion of them, ii) any claim of a breach of any of warranty or representation by Client, or iii) any claim of personal injury or property damage to the extent that the injury or damage arises other than from a breach of the relevant Product Agreement by Patheon, including, without limitation, any representation or warranty contained in this Agreement, except to the extent that the losses, damages, costs, claims, demands, subpoenas, judgments, and liability are due to the negligence or wrongful acts of Patheon, its officers, employees, or Affiliates.

If a claim occurs, Patheon will: (i) promptly notify Client of the claim; (ii) use commercially reasonable efforts to mitigate the effects of the claim; (iii) reasonably cooperate with Client in the defense of the claim; and (iv) permit Client to control the defense and settlement of the claim, all at Client's cost and expense; provided that Patheon consents in writing to any settlement..

10.5 Reasonable Allocation of Risk.

This Agreement (including, without limitation, this Section 10) is reasonable and creates a reasonable allocation of risk for the relative profits the parties each expect to derive from the Product assuming the Product is correctly manufactured, stored and delivered by Patheon in accordance with the terms and conditions of this Agreement. Patheon assumes only a limited degree of risk arising from the manufacture, distribution, and use of the Product because Client has developed and holds the marketing approval for the Product, Client requires Patheon to manufacture and label the Product strictly in accordance with the Processing Instructions, and Client, not Patheon, is best positioned to inform and advise potential users about the circumstances and manner of use of the Product.

10.6 Validation Batches.

Where Product is manufactured by Patheon (or any of its Affiliates) under a separate pharmaceutical development or technology transfer agreement (the "**Development Agreement**") and then released by Patheon for commercial sale or distribution by Client, the performance of the applicable pharmaceutical development or technology transfer services including the manufacture of the Product will be governed by the terms of the Development Agreement and will not be subject to the terms and conditions of this Agreement. The terms of this Agreement and the applicable Product Agreement will apply to any Product after release by Patheon.

11. Confidentiality

11.1 Confidential Information.

"**Confidential Information**" means any and all information disclosed by the Disclosing Party to the Recipient (whether disclosed in oral, written, electronic or visual form) that is non-public, confidential or proprietary including, without limitation, information relating to the Disclosing Party's patent and trademark applications, process designs, process models, drawings, plans, designs, data, databases and extracts therefrom, formulae, methods, know-how and other intellectual property, its clients and its clients' confidential information, finances, marketing, products and processes and all price quotations, manufacturing or professional services proposals, information relating to composition, proprietary technology, and all other information relating to manufacturing capabilities and operations. In addition, all analyses, compilations, studies, reports or other documents prepared by any party's Representatives containing Confidential Information will be considered Confidential Information. Samples or materials provided under this Agreement as well as any and all information derived from the approved analysis of the samples or materials will also constitute Confidential Information. A party's rights and obligations under this Section 11 will apply to any Confidential Information that is disclosed by or received by that party's Representatives. For the purposes of this Section 11, a party receiving Confidential Information under this Agreement (including through its Representatives) is a "**Recipient**", and a party disclosing Confidential Information under this Agreement (including through its Representatives) is the "**Disclosing Party**". The existence, parties to, and terms of this Agreement or of any Product Agreement will be considered Confidential Information.

11.2 Use of Confidential Information.

The Recipient will use the Confidential Information solely for the purpose of meeting its obligations under this Agreement or a related agreement. The Recipient will now, and in the future, keep the Confidential Information strictly confidential and will not disclose the Confidential Information in any manner whatsoever, in whole or in part, other than to those of its Representatives who: (i) have a need to know the Confidential Information for the purpose of this Agreement; (ii) have been advised of the

confidential nature of the Confidential Information and (iii) have obligations of confidentiality and non-use to the Recipient no less restrictive than those of this Agreement. Recipient will protect the Confidential Information disclosed to it by using reasonable precautions to prevent the unauthorized disclosure, dissemination or use of the Confidential Information, which precautions will not be less than those exercised by Recipient for its own confidential or proprietary Confidential Information of a similar nature.

11.3 Exclusions.

The obligations of confidentiality in this Section 11 will not apply to the extent that Confidential Information:

(a) is or becomes publicly known through no breach of this Agreement or fault of the Recipient or its Representatives;

(b) is in the Recipient's possession at the time of disclosure by the Disclosing Party, as evidenced by the Recipient's competent written records, other than as a result of the Recipient's breach of any legal obligation;

(c) is or becomes known to the Recipient on a non-confidential basis through disclosure by sources, other than the Disclosing Party, having the legal right to disclose the Confidential Information, if the other source is not known by the Recipient to be bound by any obligations (contractual, legal, fiduciary, or otherwise) of confidentiality to the Disclosing Party for the Confidential Information;

(d) is independently developed by employees, advisors or consultants of the Recipient not involved with Client and without use of or reference to the Disclosing Party's Confidential Information as evidenced by Recipient's competent written records; or

(e) is expressly authorized for release by the written authorization of the Disclosing Party.

Any combination of information which comprises part of the Confidential Information is not exempt from the obligations of confidentiality merely because individual parts of that Confidential Information are covered by exceptions in this Section 11.3, unless the combination itself is covered by any of those exceptions.

11.4 Photographs and Recordings.

Neither party will take any photographs or videos of the other party's facilities, equipment or processes, nor use any other audio or visual recording equipment (such as camera phones) while at the other party's facilities, without that party's express written consent.

11.5 Permitted Disclosure.

Notwithstanding any other provision of this Agreement, the Recipient may disclose Confidential Information of the Disclosing Party to the extent required, as advised by counsel, in response to a valid order of a court or other governmental body or as required by law, regulation or stock exchange rule. But the Recipient will advise the Disclosing Party in advance of the disclosure and limit the required disclosure to the extent practicable and permissible by the order, law, regulation or stock exchange rule and any other applicable law, will reasonably cooperate with the Disclosing Party, if required, in seeking an appropriate protective order or other remedy, and will otherwise continue to perform its obligations of confidentiality set out in this Agreement. If any public disclosure is required by law, the parties will consult concerning

the form of announcement prior to the public disclosure being made. Further, the Client shall be entitled to disclose Confidential Information to any Regulatory Authority, investors and potential investors, landlords, financing parties; including investment bankers, securities regulators, current officers, directors, stockholders, potential collaborators; provided, however, that if any such third party: (i) refuses to sign a Confidentiality Agreement with terms substantially similar to the terms in this Article 11 and (ii) is requesting Patheon Confidential Information , then Client agrees that it will not disclose such Confidential Information to the third party without first notifying Patheon and the Parties mutually agreeing to the process for the disclosure of such information.

11.6 Marking.

The Disclosing Party will use reasonable efforts to summarize in writing the content of any oral disclosure or other non-tangible disclosure of Confidential Information within [***] of the disclosure, but failure to provide this summary will not affect the nature of the Confidential Information disclosed if the Confidential Information was: (i) identified as confidential or proprietary when disclosed orally or in any other non-tangible form and (ii) given the nature of the information, should in the normal course, be considered and treated as Confidential Information.

11.7 Return of Confidential Information.

Upon the written request of the Disclosing Party, the Recipient will promptly return the Confidential Information to the Disclosing Party or, if the Disclosing Party directs, destroy all Confidential Information disclosed in or reduced to tangible form including any copies, summaries, compilations, analyses or other notes derived from the Confidential Information except for one (1) copy which may be maintained by the Recipient for its records for archival purposes and to ensure compliance with the terms and conditions of this Agreement. The retained copy will be retained by general counsel, or designee, in a secure environment and will, at all times, remain subject to all confidentiality provisions contained in this Agreement.

11.8 Remedies.

The parties acknowledge that monetary damages may not be sufficient to remedy a breach by either party of this Section 11 and agree that the non-breaching party will be entitled to seek specific performance, injunctive or other equitable relief to prevent breaches of this Section 11 and to specifically enforce Section 11 in addition to any other remedies available at law or in equity. These remedies will not be the exclusive remedies for breach of this Section 11 but will be in addition to any and all other remedies available at law or in equity.

12. **Intellectual Property**

12.1 Inventions.

(a) For the term of this Agreement as defined in Section 8.1, Client grants to Patheon a non-exclusive, fully paid-up, royalty-free, non-transferable and non-sublicensable (except to the extent required for any agreed upon subcontracted services) license of Client's Intellectual Property which Patheon must use in order to perform the Manufacturing Services.

(b) All Client Intellectual Property will be and remain the exclusive property of Client.

(c) All Patheon Intellectual Property will be the exclusive property of Patheon. Unless Patheon identifies in advance any specific Patheon Intellectual Property that will be subject to a separate licensing agreement between the parties and Client consents in advance to use of such Intellectual Property, Patheon hereby grants to Client a non-exclusive, perpetual, fully-paid-up, royalty-free, transferable license with the right to grant sublicenses through all tiers of the Patheon Intellectual Property used by Patheon in the manufacture of the Product for use in relation to manufacturing that Product and as needed for the commercialization and sale of the Product.

(d) Each party will be solely responsible for the costs of filing, prosecution, and maintenance of patents and patent applications on its own Inventions.

(e) Either party will give the other party written notice, as promptly as practicable, of all Inventions which can reasonably be considered to be improvements or other modifications of the Product, processes or technology owned or otherwise controlled by the party.

12.2 Intellectual Property.

Neither party has, nor will it acquire, any interest in any of the other party's Intellectual Property unless otherwise expressly agreed to in writing. Neither party will use any Intellectual Property of the other party, except as specifically authorized by the other party or as required or permitted for the performance of its obligations under this Agreement.

13. **Miscellaneous**

13.1 Insurance.

Each party will maintain commercial general liability insurance, including blanket contractual liability insurance covering the obligations of that party under this Agreement through the term of this Agreement and for a period of [***] after that. This insurance will have policy limits of not less than: (i) USD [***] for each occurrence for personal injury or property damage liability; and (ii) USD [***] in the aggregate per annum for product and completed operations liability. Any combination of Primary and Excess Umbrella Liability policies may be utilized to maintain the required limits. If requested each party will give the other a certificate of insurance evidencing the above and showing the name of the issuing company, the policy number, the effective date, the expiration date, and the limits of liability. The insurance certificate will further provide for a minimum of [***] written notice to the insured of a cancellation of, or material change in, the insurance. If a party is unable to maintain the insurance policies required under this Agreement through no fault of its own, then the party will without delay notify the other party in writing and the parties will in good faith negotiate appropriate amendments to the insurance provision of this Agreement in order to provide adequate assurances.

13.2 Independent Contractors.

The parties are independent contractors and this Agreement, and any Product Agreement does not create between the parties any other relationship such as, by way of example only, that of employer and employee, principal and agent, joint-venturers, co-partners, or any similar relationship, the existence of which is expressly denied by the parties.

13.3 No Waiver.

Neither party's failure to require the other party to comply with any provision of this Agreement or any Product Agreement will be considered a waiver of the provision or any other provision of this Agreement or any Product Agreement, with the exception of Sections 6.1 and 8.2 of this Agreement.

13.4 Assignment.

(a) Patheon may not assign this Agreement or any Product Agreement or any of its associated rights or obligations without the written consent of Client, this consent not to be unreasonably withheld.

(b) Subject to Section 8.2(e), Client may assign this Agreement or any Product Agreement or any of its associated rights or obligations without approval from Patheon. However, Client agrees that it will give Patheon prior written notice of any assignment, and any assignee will covenant in writing with Patheon to be bound by the terms of this Agreement or the Product Agreement, and Client will remain liable under this Agreement Any Partial Assignment will be subject to Patheon's cost review of the assigned Product and Patheon may terminate this Agreement or any Product Agreement or any assigned part of them, on [***] prior written notice to Client and the assignee if good faith discussions do not lead to agreement on amended Manufacturing Service fees within a reasonable time. Client will reimburse Patheon for any reasonable costs incurred by Patheon in connection with the Partial Assignment including any expenses incurred by Patheon for any due diligence audits in connection with the Partial Assignment.

(c) Despite the preceding provisions of this Section 13.4, either party may assign this Agreement or any Product Agreement, without any consent of approval of the other Party, but upon written notice to the other Party, to any of its Affiliates or to a successor to or purchaser of all or substantially all of its business or equity securities or pursuant to a merger, consolidation, acquisition or other business combination, provided the assignee executes an agreement with the non-assigning party whereby the assignee agrees to be bound by the obligations of this Agreement.

13.5 Force Majeure.

Neither party will be liable for the failure to perform its obligations under this Agreement or any Product Agreement if the failure is caused by an event beyond that party's reasonable control, consisting of: strikes or other labor disturbances, lockouts, riots, quarantines, communicable disease outbreaks, wars, acts of terrorism, cyber-attacks, fires, floods, storms, interruption of or delay in transportation, lack of or inability to obtain fuel, power or components, or compliance with any order, regulation, or enforcement decision of any government entity (a "**Force Majeure Event**"). A party claiming a right to excused performance under this Section 13.5 will immediately notify the other party in writing of the extent of its inability to perform, which notice will specify the event beyond its reasonable control that prevents the performance and only so long as the Force Majeure event exists. Neither party will be entitled to rely on a Force Majeure Event to relieve it from an obligation to pay money (including any interest for delayed payment of an undisputed amount) which would otherwise be due and payable under this Agreement or any Product Agreement. If due to Force Majeure Event, Patheon is unable to supply Client with the Product for a period exceeding [***] then Client shall have the right to terminate this Agreement without further cost and with immediate effect and upon written notice to Patheon. At the end of the first [***] during the persistence of the Force Majeure Event Patheon shall reasonably determine whether it will be able to resume supplying Product at the end of such [***] period and notify Client in writing of such assessment. If Patheon has concluded that it will be unable to resume supplying Product at the end of such period Client shall have the right to terminate this Agreement without cost. Patheon will provide all reasonable assistance during the technology transfer for the Product upon termination due to force majeure per Section 8.4.

13.6 Additional Product and Services.

Additional Product may be added to, or existing Product deleted from, any Product Agreement by amendment to the Product Agreement including its Schedules as applicable. If Client requests services other than those expressly set out in this Agreement or in any Product Agreement (such as qualification of a new packaging configuration or shipping studies, or validation of alternative batch sizes), or any cost items that are specifically excluded from the Price, Patheon will provide a written quote of the fee for the additional services and Client will advise Patheon whether it wishes to have the additional services performed by Patheon. The scope of work and fees will be agreed in writing by the parties.

13.7 Notices.

All notices under this Agreement, a Product Agreement, Quarterly Agreement and other agreement shall be in writing. Further, unless otherwise agreed in a Product Agreement, any notice, approval, instruction or other written communication required or permitted under this Agreement will be sufficient if made or given to the other party by personal delivery or confirmed receipt email or by sending the same by first class mail, postage prepaid to the respective addresses or email addresses set out below:

If to Client:

Amylyx Pharmaceuticals, Inc.
43 Thorndike Street
Cambridge, MA 02141
Attention: [***]
Email address: [***]

With a copy, which shall not constitute notice, to:

Rubin and Rudman LLP
53 State Street, 15th Floor
Boston, MA 02109
Attn: [***]
Email address: [***]

If to Patheon:

PATHEON INC.
111 Consumers Road
Whitby, ON L1N 5Z5
Attention: [***]
Email address:
With a copy to:

Attention: [***] (at the same address)

or to any other addresses or email addresses given to the other party in accordance with the terms of this Section 13.7. Notices or written communications made or given by personal delivery, or email will be considered to have been sufficiently made or given when sent (receipt acknowledged), or if mailed, five days after being deposited in the United States, Canada, or European Union mail, postage prepaid or upon receipt (supported by reasonable written evidence), whichever is sooner.

13.8 Severability.

If any provision of this Agreement or any Product Agreement is determined by a court of competent jurisdiction to be invalid, illegal, or unenforceable in any respect, that determination will not impair or affect the validity, legality, or enforceability of the remaining provisions, because each provision is separate, severable, and distinct.

13.9 Entire Agreement and Amendment.

This Agreement, together with its Appendices, the applicable Product Agreement, Capital Equipment Agreement (if any), and the Quality Agreement, constitutes the full, complete, final and integrated agreement between the parties relating to the subject matter of the Agreement and supersedes all previous written or oral negotiations, commitments, representations, agreements, transactions, or understandings concerning the subject matter of this Agreement. The basis of the parties' agreement is set out expressly and they have not been induced by or relied on any statement or representation that is not set out in this Agreement. Any modification, amendment, or supplement to this Agreement or any Product Agreement must be in writing and signed by authorized representatives of both parties. In case of conflict, the prevailing order of documents will be this Agreement, the Product Agreement, and the Quality Agreement (except that the Quality Agreement will prevail in relation to quality matters).

13.10 Other Terms.

No terms, provisions or conditions of any purchase order or other business form or written authorization used by the parties will have any effect on the rights, duties, or obligations of the parties under or otherwise modify this Agreement or any Product Agreement, regardless of any failure of a party to object to the terms, provisions, or conditions unless the document specifically refers to this Agreement or the applicable Product Agreement and is signed by both parties.

13.11 No Third Party Benefit or Right.

Nothing in this Agreement or any Product Agreement will confer or be construed as conferring on any third party any benefit or the right to enforce any express or implied term of this Agreement or any Product Agreement (except that Patheon Affiliates acting as subcontractors under this Agreement may enforce Sections 10.1 and 10.2). The rights of the parties to terminate, rescind or agree any variation, waiver or settlement under this Agreement are not subject to the consent of any other person.

13.12 Execution in Counterparts.

This Agreement and any Product Agreement may be executed in two or more counterparts, by original or electronic (including "**pdf**") signature, each of which will be considered an original, but all of which together will constitute one and the same instrument.

13.13 Use of Name.

Neither party may use the other party's name, trademarks or logo or any variations of them, alone or with any other word or words, without the prior written consent of the other party. Despite this, Client agrees that Patheon may include Client's name and logo in customer lists or related marketing and promotional material for the purpose of identifying users of Patheon's Manufacturing Services.

13.14 Taxes.

(a) VAT.

Any payment due to Patheon under this Agreement in consideration for the provision of Manufacturing Services to Client by Patheon is exclusive of value added taxes ("**VAT**"), turnover taxes, sales taxes or similar taxes, including any related interest and penalties (together referred to as "**Transaction Tax**"). If any Transaction Tax is payable on a Manufacturing Service supplied by Patheon to Client under this Agreement, this Transaction Tax will be added to the invoice amount and will be for the account of (and reimbursable to Patheon by) Client.

If any Transaction Tax on the supplies by Patheon is payable by Client under a reverse charge or withholding procedure (i.e., shifting of liability, accounting or payment requirement to recipient of supplies), Client will ensure that Patheon will not effectively be held liable for this Transaction Tax by the relevant taxing authorities or other parties.

Where applicable, Patheon will use its reasonable commercial efforts to ensure that its invoices to Client are issued in a way to meet the requirements for deduction of input VAT by Client, if Client is permitted by law to do so.

Each party will provide the other with reasonable assistance to enable the recovery, as permitted by Applicable Laws, of Transaction Tax resulting from payments made under this Agreement, this recovery to be for the benefit of the party bearing the Transaction Tax.

If Patheon is acting as Client's buying agent, Patheon will always charge to Client the Transaction Tax in the relevant territory in addition to the amount paid by Patheon to supplier.

For the avoidance of doubt, reference to the Manufacturing Services in this Section also includes any element (or the entirety) of the Manufacturing Services characterized as a supply of goods by Patheon, its subcontractors or any tax authority for Transaction Tax purposes.

(b) Duties.

Client will bear the cost of all duties, levies, tariffs and similar charges (and any related interest and penalties) (together "**Duties**") however designated, arising from the performance of the Manufacturing Services by Patheon, including (without limitation) those imposed as a result of the shipping of materials (including drug substance, materials, components and finished Product) to, from or between Patheon sites. If these Duties are incurred by Patheon, then Patheon will be entitled to invoice Client for these Duties at the time that they are incurred.

(c) Withholding Tax.

Where any sum due to be paid to Patheon hereunder is subject to any withholding or similar tax, Client will pay the withholding or similar tax to the appropriate Government Authority without deduction from or offset of the amount then due to Patheon. The Parties agree to cooperate with one another and use reasonable efforts to reduce or eliminate or enable the recovery of any tax withholding or similar obligations in respect of royalties, milestone payments, and other payments made by Client to Patheon under this Agreement.

Patheon will provide Client any tax forms that may be reasonably necessary in order for Client not to withhold tax or to withhold tax at a reduced rate under an applicable bilateral income tax treaty.

Each Party will provide the other with reasonable assistance to enable the recovery, as permitted by Applicable Laws, of withholding taxes, or similar obligations resulting from payments made under this Agreement, this recovery to be for the benefit of the Party bearing the withholding tax.

(d) No Offset. Any Transaction Tax, Duty, Withholding Tax or other tax that Client pays, or is required to pay, but which Client believes should properly be paid by Patheon under this Agreement may not be offset against sums due by Client to Patheon whether due under this Agreement or otherwise.

13.15 Governing Law and Jurisdiction.

This Agreement and any Product Agreement, and any dispute or claim (including non-contractual disputes or claims) arising out of or in connection with them or their subject matter or formation are governed by the laws of the State of Delaware without regard to any conflicts-of-law principle that directs the application to another jurisdiction's law. Both parties hereby submit to the exclusive jurisdiction of the courts located in the State of Delaware. The parties further expressly agree that the UN Convention on Contracts for the International Sale of Goods will not apply to this Agreement.

13.16 Dispute Resolution.

All disputes that arise under or in connection with this Agreement will be resolved in accordance with Appendix 2.

SIGNATURE PAGE TO FOLLOW

This Agreement is signed by the authorized representatives of the parties on the dates shown below and will take effect from the Effective Date.

PANTHEON INC.

By: /s/ Don Liscombe
Name: Don Liscombe
Title: General Manager, Commercial Operations
Date: November 19, 2019

AMYLYX PHARMACEUTICALS, INC.

By: /s/ Justin Klee
Name: Justin Klee
Title: President
Date: November 12, 2019

APPENDIX 1—Form of Product Agreement

Product Agreement for [INSERT PRODUCT NAME]

This Product Agreement (this "**Product Agreement**") is issued under the Master Manufacturing Services Agreement dated October 1, 2019 between PATHEON INC. and AMYLYX PHARMACEUTICALS, INC., (the "**Master Agreement**"), and is entered into on [INSERT DATE] (the "**Effective Date**") between [PATHEON ENTITY], a corporation existing under the laws of [ ], having a principal place of business at [PATHEON ENTITY ADDRESS] ("**Patheon**") and Amylyx Pharmaceuticals, Inc., a Delaware corporation having a principal place of business at 43 Thorndike Street, Cambridge, MA 02141 ("**Client**"). For the purpose of this Product Agreement, references in the Master Agreement to "Patheon" and "Client" mean the entities defined respectively as Patheon and Client in this Product Agreement.

The terms and conditions of the Master Agreement are incorporated into this Product Agreement except to the extent this Product Agreement expressly modifies specific provisions in the Master Agreement. All capitalized terms that are used but not defined in this Product Agreement will have the respective meanings given to them in the Master Agreement.

1. **Initial Product Term**: will be from the Effective Date until December 31, 20[ ]
2. **Manufacturing Site**: The Manufacturing Services will be performed at the following Manufacturing Site: [ ]
3. **Minimum Market Requirement**: (if different from the Master Agreement, capture Launch Period and period following the Launch Period)
4. **Notices**: (if different from Section 13.7 of the Master Agreement): [insert contact details]
5. **API Name**: [insert API name]
6. **API Credit Value**: Client's actual cost for API not to exceed [ ] per kilogram. API value to be provided by Client and supported by such reasonable evidence as Patheon requests.
7. **Local Currency**: [insert currency]
8. **Billing Currency**: [insert currency]
9. **Initial Exchange Rate**: [1.00 Local Currency] to [1.00 Billing Currency]
10. **Inflation Index**: [if different from the Master Agreement]
11. **Governing Law**: [if different from the Master Agreement]]
12. **Other Modifications to the Master Agreement (if any)**: [insert here; for currencies not in EURO or USD, insert the following language: The parties agree that for purposes of this Product Agreement, all references in the Master Agreement to EURO/USD are hereby converted to [CAD] at the exchange rate of 1 EURO/USD to x [CAD].]

Schedule A - Commercial Supply Pricing Proposal: Description of the Manufacturing Services and related terms of this Product Agreement, which may include: Product Features and Assumptions, Key Assumptions to be Finalized, Annual Volume Forecasts, Pricing Tables, Costs Included in Price, Costs Not Included in Price, Equipment Requirements (if applicable), Manufacturing Parameters, Packaging Parameters, Testing Conditions, Supply Chain.

In case of conflict between Schedule A and the other parts of this Product Agreement, those other parts will prevail.

This Agreement is signed by the authorized representatives of the parties on the dates shown below and will take effect from the Effective Date.

[PANTHEON ENTITY]

By: _____
Name: _____
Title: _____
Date: _____

AMYLYX PHARMACEUTICALS, INC.

By: _____
Name: _____
Title: _____
Date: _____

Schedule A - Commercial Supply Pricing Proposal

[Insert Commercial Supply Pricing Proposal]

[End of Product Agreement]

APPENDIX 2 - Dispute Resolution

Negotiation

If any dispute arises out of this Agreement or any Product Agreement, the parties will first try to resolve it amicably. Any party may send a notice of a dispute to the other, and each party will appoint, within [***] from receipt of the notice, an appropriate single representative having full power and authority to resolve the dispute. The representatives will meet as necessary in order to resolve the dispute. If the representatives fail to resolve the matter within [***] from their appointment, or if a party fails to appoint a representative as required above: for Technical Disputes, the expert determination procedure may be started by either party; and for all other disputes, each party will refer the dispute immediately to the Chief Operating Officer or equivalent (or another senior manager as he/she may designate) ("**Senior Officers**") who will meet and discuss as necessary to try to resolve the dispute amicably.

Mediation

If the Senior Officers fail to resolve the dispute, the parties will attempt in good faith to settle the dispute promptly by confidential mediation under the then current CPR Mediation Procedure, before resorting to litigation. If one party fails to participate in settlement negotiations as provided in this Appendix 2, the other party may initiate mediation prior to the expiration of the applicable negotiation periods. The mediator will be chosen with the assistance of CPR (and CPR's choice will be accepted by the parties in the absence of conflict or bias), unless the parties agree on a specific mediator in writing within [***] of the referral to mediation. The mediation will take place in Boston, Massachusetts and the language of the mediation will be English. Unless otherwise agreed, the parties will select a mediator from the CPR Panels of Distinguished Neutrals.

Except where proceedings are required for the purpose of an interim injunction or other interim equitable relief or to preserve a party's legal position following the outcome of negotiation or mediation, neither party may commence any court proceedings in relation to a dispute until the required mediation has ended without resolving that dispute or a party fails to participate in that mediation. Where a party decides not to take part in mediation in contravention of this Appendix 2, it will send written notice of that decision to the other party.

Technical Disputes

If a dispute arises between the parties that is exclusively related to technical aspects of the manufacturing, packaging, labelling, quality control testing, handling, storage, or other activities under this Agreement, including conformance of Product to applicable specifications (a "**Technical Dispute**"), the parties will use all reasonable efforts to resolve the dispute by amicable negotiations as provided above. If the parties are unable to resolve a Technical Dispute by negotiation, the Technical Dispute will, at the written request of either party, be referred for determination to an expert in the following manner:

(a) Appointment of Expert. Within [***] after the written request, the parties will appoint a single agreed expert with experience and expertise in the subject matter of the dispute. If the parties fail to agree the appointment within that period, then either party may request that a neutral from the International Institute of Conflict Prevention and Resolution appoints a suitable expert (and both parties will accept that appointment in the absence of evident conflict or bias). As a condition of the expert's appointment, the parties will ensure that the expert agrees to disclose any actual or potential conflicts of interest promptly as they arise. The parties do not intend that the expert acts as an arbitrator.

(b) Procedure. The parties will require the expert to provide an opinion on each referred issue (with reasonably detailed reasoning) within [***] (or as agreed by the parties with the expert). Each party will give to the expert all the evidence and information within their respective possession or control as the expert may reasonably request, which they will disclose promptly and in any event within [***] of a written request from the expert to do so. At all times the parties will co-operate in good faith and seek to narrow and limit the issues to be determined.

(c) Final and Binding. The determination of the expert will, except for fraud or manifest error or where an unapproved conflict of interest is discovered, be final and binding upon the parties with respect to the referred Technical Dispute.

(d) Costs. Each party will bear its own costs for any matter referred to an expert under this Appendix 2 and, in the absence of express agreement to the contrary, the costs and expenses of the expert will be shared equally by the parties.

APPENDIX 3 - API Yield Calculation

Actual Annual Yield

Reconciliation: For each Year, Patheon will prepare an annual reconciliation of API including the calculation of the Actual Annual Yield as set forth below.

"**Actual Annual Yield**" means the percentage of the Quantity Dispensed that was converted to Products for the Product at the Manufacturing Site in that Year and is calculated as follows:

$$Actual\ Annual\ Yield\ (\%) = \frac{\text{Quantity Converted}}{\text{Quantity Dispensed}} \times 100$$

"**Quantity Dispensed**" means the API received and dispensed in commercial manufacturing of Product, calculated as follows:

The total quantity of API that complies with the specifications and is received at the Manufacturing Site during the Year added to the inventory of API that complies with the specifications held at the start of the Year, minus the inventory of API that complies with the specifications held at the end of the Year.

The Quantity Dispensed includes API lost in the warehouse prior to and during dispensing but excludes (i) API retained by Patheon as samples; (ii) API contained in Product retained as samples; (iii) API used in testing (if applicable); (iv) API contained in Product that is rejected for specific market related requirements such as visual inspection of the Product that is not part of normal processing and (v) API received or dispensed in technical transfer activities or development activities, including without limitation, any regulatory, stability, validation or test batches manufactured during the applicable period.

"**Quantity Converted**" means the total amount of API contained in the Product manufactured with the Quantity Dispensed (including any additional Product supplied as a replacement remedy) and released for delivery, but not rejected as Deficient Product in accordance with Section 6.1. The quantity of API contained in Deficient Product will be included in the Quantity Dispensed but not in the Quantity Converted.

Target Yield and Credit Calculation

After Patheon has produced a minimum of ten successful commercial production batches of Product and has produced commercial production batches for at least six months at the Manufacturing Site, the parties will agree on the target yield for the Product at the Manufacturing Site (as a percentage of the Quantity Dispensed to be converted to Product, this percentage a "**Target Yield**"). After the six month period, and then before the start of each subsequent Year, the parties will enter into good faith discussions to agree on a Target Yield for that Year which reasonably reflects the actual manufacturing experience of Patheon to date. Once the Target Yield is agreed, the following applies:

Shortfall Credit Calculation. If the Actual Annual Yield falls greater than [***] below the respective Target Yield in a Year, then the shortfall credit for the Year (the "**Shortfall**") will be calculated as follows:

$$Shortfall = (Target\ Yield - [***] - Actual\ Annual\ Yield) \times API\ Credit\ Value \times Quantity\ Dispensed$$

Surplus Credit Calculation. If the Actual Annual Yield is greater than the respective Target Yield in a Year, then the surplus credit for that Year (the "**Surplus**") will be determined based on the following calculation:

$$Surplus = (Actual\ Annual\ Yield - Target\ Yield) \times API\ Credit\ Value \times Quantity\ Dispensed$$

Shortfall Credit. If there is a Shortfall for a Product in a Year, then Patheon will credit Client's account for the amount of the Shortfall not later than [***] after the end of each Year.

Surplus Credit. If there is a Surplus for a Product in a Year, then Patheon will be entitled to apply the amount of the Surplus as a credit against any Shortfall for that Product which may occur in the next Year. If there is no Shortfall in the next Year the Surplus credit will expire.

Each credit under this paragraph will be summarized in an annual reconciliation report. Upon expiration or termination of a Product Agreement, any remaining Shortfall credit amount owing under this paragraph will be paid to Client.

Limits on API Liability

A Shortfall caused by rejected Deficient Product (including in the case of Recall) will only result in a Shortfall Credit to the extent the affected Product is unsold and returned, destroyed or otherwise disposed of by Client in accordance with the terms of this Agreement.

Any payable reimbursement (within the maximum liability limits) for lost API will be made at the API Credit Value.

APPENDIX 4 - Price Adjustments

Price Adjustment Calculation Due To Inflation

Refer to Section 4.2(a)

Definitions:

"**Inflation Index**" means the overall harmonised Index of Consumer Prices (HICP) published by the European Central Bank (www.ecb.europa.eu/stats/prices/hicp/html/index.en.html) for Manufacturing Sites in Europe, and the Producer Price Index pcu32541235412 for Pharmaceutical Preparation Manufacturing (PPI) published by the United States Department of Labor, Bureau of Labor Statistics (hyperlink) for Manufacturing Sites in North America.

"**Inflation Percentage**" means the average of the monthly annual percentage changes in the Inflation Index from September of the preceding Year to August of the then current Year. For example, at the end of 2019 the new Inflation Percentage would be calculated as follows (figures are for illustration only):

From: Month - Year	To: Month - Year	Annual Percentage Change
September - 2017	September - 2018	0.7%
October - 2017	October - 2018	1.1%
November - 2017	November - 2018	1.0%
December - 2017	December - 2018	0.8%
January - 2018	January - 2019	0.8%
February - 2018	February - 2019	1.1%
March - 2018	March - 2019	1.1%
April - 2018	April - 2019	1.5%
May - 2018	May - 2019	1.7%
June - 2018	June - 2019	1.4%
July - 2018	July - 2019	1.2%
August - 2018	August - 2019	1.1%
Inflation Percentage		1.13%

Calculation:

$$New\ Price = Current\ Price + (Current\ Price \times Inflation\ Percentage)$$

Price Adjustment Calculation Due To Currency Fluctuation

Refer to Section 4.2(b)

Definitions:

"**Billing Currency**" means the currency in which the Manufacturing Services will be invoiced and paid as specified in the Product Agreement.

"**Local Currency**" means the currency that is used in the country where the Manufacturing Site is located as specified in the Product Agreement.

"**Initial Exchange Rate**" means the initial exchange rate set out in the Product Agreement to convert one unit of the Patheon Manufacturing Site Local Currency into the Billing Currency for the first Year of the Product Agreement.

"**Current Year Exchange Rate**" means the exchange rate calculated as of the current Year of the Product Agreement (starting from the second year), and is calculated as the average interbank exchange rate for conversion of one unit of the Patheon Manufacturing Site Local Currency into the Billing Currency during the period (September 1st of the preceding year to August 31st of the current year) as published by OANDA.com under the heading "Average Exchange Rates" at www.oanda.com/currency/average.

"**Preceding Year Exchange Rate**" means the exchange rate calculated in the previous Year to the then current Year of the Product Agreement.

Calculation:

$$New\ Price = \frac{Current\ Price\ (after\ inflation)}{\begin{matrix}Preceding\ Year\ Exchange\ Rate \\ or\ Initial\ Exchange\ Rate\ (second\ Year\ Only)\end{matrix}} \times Current\ Year\ Exchange\ Rate$$

For example:

Billing Currency	USD
Local Currency	EURO
Current Price (after inflation)	1.50 USD
Preceding Year Exchange Rate	1.2 (1 EURO to 1.2 USD)
Current Year Exchange Rate	1.1 (1 EURO to 1.1 USD)
New Price	$= \frac{1.50\ USD}{1.2} \times 1.1$ $= 1.375\ USD$

Certain identified information has been excluded from this exhibit because it is both not material and is the type that the registrant treats as private or confidential. Information that was omitted has been noted in this document with a placeholder identified by the mark "[***]".

SUPPLY

AGREEMENT

This Supply Agreement (the "**Agreement**") dated as of October 29, 2019 (the "**Effective Date**"), is made and entered into by and between:

Amylyx Pharmaceuticals, Inc. a corporation incorporated and existing under the laws of the State of Delaware, with registered offices at 43 Thorndike Street, Cambridge, MA 02141 U.S.A., acting on Its behalf and on behalf of its Affiliates, duly represented by Thomas Holmes, Global Head, Supply Chain;

("**Buyer**")

AND

CU Chemie Uetikon, GmbH, a company incorporated and existing under the laws of Germany, with registered offices at Raiffeisenstr 4, 77933 Lahr, Germany and registered under number Freiburg HBR 390945 acting on its behalf and on behalf of its Affiliates, duly represented by Frédéric Desdouits, Managing Director;

("**SEQENS CDMO**" or "**Supplier**")

Both Supplier and Buyer are individually referred to as a "**Party**" and collectively, the "**Parties**".

PREAMBLE:

WHEREAS, SEQENS CDMO Is the division of the SEQENS group specialized in the manufacture and sale of fine chemicals and active pharmaceutical ingredients and other specialty molecules for use in the pharmaceutical and chemical industries. For the purpose of this Agreement, an "**Affiliate**" refers to any legal entity controlled by or under common control with Supplier (control as defined by the OCDE) that is actively Involved in the performance of this Agreement;

WHEREAS, Buyer is developing a product that will ultimately include the API, as hereinafter defined, to make the Buyer's finished dose formulation (the "**End Product**"). The End Product requires for its manufacture the use of the active pharmaceutical ingredient sodium phenylbutyrate ([***]) produced by Supplier at its Facility (the "**API**");

WHEREAS, Buyer wishes to secure sourcing of the API including its registration in the End Product drug master file in view of obtaining its marketing authorisation from the FDA in the United States of America;

WHEREAS, Supplier has polyvalent manufacturing capacities able to manufacture and supply the API on an industrial and commercial scale.

NOW AND THEREFORE, for good and valuable consideration, Including the herein agreements, covenants, promises, representations and warranties, the receipt and legal sufficiency of which is hereby acknowledged, accepted and agreed to, the Parties hereby agree as follows:

1. Scope

1.1 During the Term of this Agreement and in accordance with the terms and conditions of this Agreement, Buyer agrees to purchase from Supplier, on a non-exclusive basis, and Supplier represents, warrants and agrees to timely manufacture, supply and deliver to Buyer, in accordance with this Agreement, all quantities of API ordered by Buyer for the formulation, distribution and sale of the End Product. The Supplier will purchase sufficient raw materials and schedule the necessary Facilities resources and capacity by allocating a specific time period at the Facility for the manufacture of the API ordered by the Buyer. Supplier is responsible for all API activities required to be performed under this Agreement and will support all of Buyer's regulatory filings.

1.2 The API is supplied on a non-exclusive basis. Supplier shall remain at all times owner of the End Product's drug master file and this Agreement shall not prevent Supplier from manufacturing and selling API to third parties. The Supplier shall fully cooperate with the Buyer to provide all required access to the API DMF and other data that may be required by any relevant regulatory Authority (only the open part DMF will be directly provided to the Buyer) and shall provide worldwide support for the Buyer's registration and approval of the End Product.

1.3 Buyer is and shall remain the sole owner of the End Product.

2. Condition precedent and subsequent

2.1 The End Product is currently in an FDA approved clinical trial and the Buyer anticipates receiving marketing authorizations during [***] (the "**MA**") from the relevant regulatory authorities; specifically, the food and drug administration (FDA) for the USA.

2.2 Prior to reception of the MA, it is understood and agreed that Supplier will manufacture and supply the API in accordance with special purchase orders to be placed, from time to time, in accordance with the Buyer's development needs (the "**Pre-MA Purchase Order**").

2.3 Supplier shall make available to Buyer the open part of the DMF.

3. Product and volumes

3.1 Supplier shall timely manufacture, deliver and supply API in accordance with all of the specifications laid out Exhibit 1 and this Agreement (the "**Specifications**").

3.2 Supplier shall manufacture the API at its industrial site CU UETIKON located at Raiffeisenstrasse 4 - 77933, Lahr, Germany (the "**Site**"). All API shall be manufactured in accordance with cGMP (current Good Manufacturing Practice) as promulgated under the U.S. Federal Food, Drug, and Cosmetic Act at 21 CFR (chapters 210 and 211), as the same may be amended or re-enacted from time to time. As a condition precedent and prior to Buyer accepting initial delivery of the API, Supplier agrees to provide Buyer with documentation verifying that Supplier's plant has been inspected by the U.S. FDA and that it is in good standing with respect to its manufacturing practices as evidenced by an acceptable Establishment Inspection Report issued by the FDA (an "**EIR**"). Supplier shall supply promptly deliver to Buyer a copy of each EIR as issued during the Term of this Agreement.

3.3 Supplier agrees, during the Term, to obtain and maintain throughout the Term any and all U.S. governmental approvals, registrations or licenses necessary to manufacture, sell and distribute the API to Buyer; and further agrees to permit the inspection of its plant where the API is being manufactured (the "**Facility**") by the Buyer and authorized representatives of the FDA at all times.

3.4 Buyer shall order the API in multiples of [***]. All API shall meet the specifications provided by Buyer and Seller shall provide a Certificate of Analysis ("**COA**") with each delivery of API.

4. Capacity constraints and development

4.1 Supplier represents that it has a current capacity to manufacture [***] per month of API and an aggregate yearly volume of [***] of API (the "**Max Capacity**"). [Consider EX US supply & Japan). The Supplier agrees that it will maintain the Max Capacity during the Term of this Agreement. Notwithstanding anything to the contrary herein contained, the Supplier represents and warrants that it will have and it will continue to have during the "**Launch Period**", as hereinafter defined, the capacity to timely manufacture and deliver to the Buyer all API ordered by the Buyer. For purposes of this Agreement, the Launch Period is defined to mean the [***] period commencing on the Effective Date and terminating at the end of the [***] following the Effective Date.

4.2 Supplier may update this Max Capacity from time to time to take account of Buyer's actual requirements and those of eventual third party clients.

4.3 In the event that Buyer's orders for the API exceed Supplier's Max Capacity then both Parties shall discuss commercially viable solutions to increase such Max Capacity. In the event that Buyer orders exceed Supplier's Max Capacity, whether on a monthly or yearly basis, then Supplier shall only be bound to supply an amount equal to its Max Capacity as expressed above. For avoidance of doubt, it is understood and agreed that if the Buyer's requirements exceed the Supplier's Max Capacity or if Supplier is unable to supply all or any part of the API, then the Buyer, in either event, shall be entitled without any obligation to the Supplier, to purchase such excess requirements from a third party.

5. Raw materials sourcing

5.1 Supplier shall be responsible for sourcing, purchasing and supplying all raw material required for the manufacture of the API.

5.2 In the event that Supplier discovers any threat to sourcing security that relates to any activity or obligations of the Buyer, then it shall immediately inform Buyer and Buyer shall reasonably cooperate for the resolution of the issues (such as any regulatory requirement affecting the End Product to qualify a second source).

6. Forecast

6.1 Supplier will be manufacturing the API in multipurpose production Facilities at the Site as such it is understood that the Facilities are used for multiple products and proper planning is required to ensure than the Facilities are adequately available and dedicated to Buyer's needs.

6.2 Buyer will therefore provide a [***] rolling forecast updated on a [***] basis. Such forecast shall be non-binding upon the Parties provided, however, that the forecast for the then [***] shall be considered a firm commitment to purchase the quantities of API set forth in such forecast and Supplier represents that the Facilities will have sufficient capacity and raw materials to supply API, in accordance with this Agreement for at least [***]. The Buyer shall provide an executed purchase order with the applicable quantity forecast and upon other terms and conditions set forth in the Purchase Order

6.2.1 Supplier shall not be required to accept any Purchase Orders (as the term is defined hereunder) in excess of the rolling forecast, but Supplier will use commercially reasonable efforts to accept and complete such Purchase Orders;

6.2.2 All Purchase Orders shall be placed in accordance with the relevant lead times;

6.2.3 The forecast may not exceed the defined Max Capacity, unless otherwise agreed to;

6.2.4 Option to reserve Supplier capacity. In the event that Buyer wishes to reserve a certain capacity of the production facilities ("**Reserved Capacities**") for delivery of API by a specific date it shall have the option, instead of placing a Purchase Order for the API, to place a Capacity Reservation that it shall have the right to cancel, in part or whole, up to [***] before the scheduled production date. In the event that it does not take all Reserved Capacities, then It shall pay an amount equal to [***] percent ([***]) of the non-used Reserved Capacities. For the avoidance of doubt, such Reserved Capacities shall be expressed in a number of kilograms of API to be produced in a defined month (subject to Max Capacity).

7. Purchase Orders

7.1 Buyer shall place orders for API in accordance with this Agreement and Supplier constraints as expressed hereunder (a "**Purchase Order**"). Supplier's form of a Purchase Order is annexed hereto as Exhibit 4 and made a part hereof as if set out verbatim.

7.2 Such Purchase Order shall be placed with a lead time of [***] prior to the requested delivery date.

7.3 Supplier shall accept or reject any Purchase Order placed in accordance with the Forecast within [***] of receipt; and if not timely rejected in writing within the aforesaid [***] period ([***]), the Purchase Order shall be deemed automatically accepted without the need for any further action or deed. In the event that Buyer places Purchase Orders in excess of the Max Capacity or the Forecast then Supplier shall, subject to Section 4, make reasonable commercial efforts to accept such Purchase Orders subject to it adjusting the times and pricing as may be required to meet such additional demand,

7.4 Upon acceptation by Supplier, the Purchase Order will become firm and binding between the Parties. In the event of a conflict between the terms of this Agreement and a Purchase Order then the terms of this Agreement shall control for all purposes unless otherwise agreed to by the Parties In writing in the Purchase Order. Furthermore any standard terms and conditions sent with or attached to a Purchase Order, its acceptance, a bill of lading, an invoice or any other standard document issued by a Party shall not apply to this Agreement,

8. Intentionally Deleted.

9. Second Site qualification

9.1 Buyer shall have the option to request that Supplier qualify a second Site and Facility for production of the API for business continuity planning subject to the following provisions.

9.2 Supplier and Buyer shall cooperate fully for the qualification of a second Site and Facility or production line in order to ensure continuous production of the API and to mitigate the impact from unforeseen circumstances.

9.3 Buyer and Supplier shall provide all requisite regulatory support to the other party for the registration of such second Site and Facility in order to ensure that Supplier may produce the API at the second Site and Facility and that Buyer may use such API in its End Product.

9.4 Upon all regulatory qualification and approval of the second Site and Facility, it is understood and agreed that Supplier may provide API from either of the Sites and Facility as may be practical for planning purposes.

9.5 It is understood that for the purpose of the second Site and Facility qualification, Buyer shall pay for and take delivery of all validation batches.

9.6 Upon qualification of the second Site and Facility, Site and Facility shall be considered in the same manner as the Site defined in Section 3.3 hereof. Supplier shall be free, for the purpose of an establishing an efficient production planning, to produce any ordered API at either Site, provided, the selection of the Site is consistent with Buyer's Purchase Order(s) and delivery terms.

10. Price & Payment

10.1 The API price is as defined in **Exhibit 2** (the "**Price**") annexed hereto and made a part hereof.

10.2 Unless otherwise mutually agreed to, the Parties will discuss and finalize any new Price increase prior to November 30th of each year applicable to the following calendar year, and effective as of January 1st of each year during the term of this Agreement.

10.3 The Price shall be inclusive of the applicable INCOTERM and exclusive of any taxes and charges including without limitation VAT. In the event that delivery is required to a place not specified in this Agreement or with a different INCOTERM, then Supplier reserves the right to adjust the Price accordingly. Currently the INCOTERM is an FCA Supplier's factory in Lahr.

10.4 Supplier shall invoice Buyer upon delivery of the API. In the event that the Invoice is sent by email with appropriate verification of receipt, then the date of delivery shall be considered the date of invoice for the purposes of this Agreement.

10.5 In the event that Supplier fulfils a Purchase Order in several instalments then it shall invoice per instalment or upon final instalment.

10.6 Buyer shall pay each undisputed invoice within [***] net from the date of receipt of invoice. In the event of any dispute, the Parties shall work together, in good faith, and use their best efforts to resolve any dispute.

10.7 Payment shall be made In EUROS by wire transfer to Supplier's bank account IBAN:

10.8 Neither Party shall have any right of set-off against the other Party for any undisputed invoice,

11. Delivery

11.1 Supplier shall deliver the API in accordance with a FCA INCOTERM at Site (Lahr, Germany) (INCOTERMS 2010 as published by the ICC).

11.2 Delivery shall occur in accordance with the Purchase Order by the date specified therein

11.3 Supplier shall ensure the API is properly labelled and packaged for transportation, in accordance with any specifications agreed upon to prevent damage to the API through ordinary handling. The API shall be accompanied by all requisite documentation except where such documentation may be send separately or electronically.

11.4 Supplier shall provide a certificate of conformity and a COA for each batch included in a delivery.

11.5 Delivery may occur in one or more instalments as agreed to by the Parties.

12. Passage of risk and title

12.1 Risk in the API shall pass in accordance with the applicable INCOTERM.

12.2 Title to the API shall pass upon payment in full of all undisputed monies owed by Buyer to Supplier; provided, however, that the Buyer may use the API without limitation, prior to the date that title passes to the Buyer. In the event of a dispute, the matter shall be resolved in accordance with Section 10,6 hereof. Until such time, Buyer shall store the API in a separated area at its warehouse and clearly identify the API as Supplier's property. In the event of insolvency proceedings, of any event of termination of this Agreement, the Buyer shall provide access to its premises for recovery of the API.

13. Reception and conformity

13.1 Upon delivery of the API to the Buyer, the Buyer shall carry out visual inspection of the API to determine whether they have been damaged during transportation, that the requisite quantities are included and that the API is accompanied by the appropriate documentation (the "**Visual Inspection**"). For the avoidance of doubt, this Visual Inspection shall include in its scope any physical defect that is readily apparent without having to unpack the Product or open its containing vessel, Supplier's failure to meet the requirements under the Visual Inspection shall be a "**Visual Defect**". In the event that a Visual Defect is observed, Buyer shall, within [***] of the Visual Inspection, provide Supplier with reasonable proof of the Visual Defect (i.e., photographs of such Visual Defect or other such documentation).

13.2 Buyer shall carry out batch testing prior to using the API and in any event within [***] of delivery Buyer shall notify Supplier within [***] of receipt of written documentation of such testing of any batch testing failure and provide, upon request, all relevant documentation, testing information and samples ("**Testing Failure**"). For the avoidance of doubt a Testing Failure occurs when the API fails to meet any of the specifications.

13.3 In the event that Buyer notifies Seller of a Testing Failure then, upon Supplier's written request, the API shall be tested by an independent qualified party (chosen by Buyer from one of three (3) proposed by Supplier unless commonly agreed). If the Testing Failure is not confirmed, Buyer shall pay for costs related to such testing. If the Testing Failure is confirmed, Supplier shall pay for costs related to such testing. In the event of such confirmation, or if Supplier accepts Buyer's claim then Supplier shall at Buyer's option: (i) replace the API free of charge in a timely manner or (ii) reimburse Buyer in full for monies paid for the API. It is understood that in the event that a Testing Failure may be remedied in such a manner as to render the API fit for use in compliance with its Specifications then Supplier shall carry out such remedy and shall not be required to complete any further reimbursement or replacement (e.g. if the COA was omitted and is then sent subsequently).

14. Quality Agreement and cGMP requirements

14.1 "**cGMP**" means current Good Manufacturing Practices as established by ICH Q7 and applied by various competent health and medical agencies around the world (such as the EMA, the FDA etc.). Supplier shall manufacture the API in accordance with cGMP regulations. Both Parties shall cooperate with each other to ensure that the other Party is aware of the territory of use of the API (or distribution) and applicable jurisdictions / competent authorities with regards to the API.

14.2 The Parties shall negotiate in good faith and execute a quality agreement (as required under cGMP) establishing the roles and responsibilities of the Parties relating to the quality and regulatory aspects of the API (the "**Quality Agreement**"). The Parties may consider and execute intermediary quality agreements prior to the MA with a definitive quality agreement being implemented subsequently.

14.3 The Quality Agreement shall be attached hereto upon execution and thereafter such Quality Agreement shall supercede any conflicting term in this Article 14

14.4 Supplier will further cooperate with Buyer for the organization of quality audits required under cGMP rules ("**Quality Audits**"). Such Quality Audits will take place on a yearly basis other than a '*for cause*' Quality Audit which may occur at any time. Quality Audits may not exceed [***] past which Supplier shall recharge an amount equal to [***] per additional day.

14.5 Buyer shall provide Supplier with reasonably advanced notice of its proposed Quality Audit and shall discuss appropriate dates with Supplier (it being understood that Supplier is running a multipurpose facility and may be already engaged for an audit for a different client). Buyer shall further execute, and have executed by its designated auditors, any required confidentiality agreements, in the usual form and format, prior to access to the Site.

15. Subcontracting

15.1 Supplier shall not subcontract the manufacture of the API to a third party and the API shall at all times be manufactured at a Supplier Site.

15.2 Notwithstanding the foregoing, it is understood and agreed that various secondary services may be contracted out to specialized third parties at a Supplier Site (such as analytical services, maintenance, engineering etc.). Such services contracted out to third parties shall not be considered as 'subcontracting' and shall not require any consent from Buyer provided the API continues to be manufactured in accordance with all of the Specifications at the Site which shall remain under Supplier's control and ownership and for which the Supplier shall remain fully responsible for the API's compliance with all of the terms and conditions of this Agreement, all Specifications and any Exhibit.

16. Intellectual property

16.1 All of the intellectual property on or related to the API and its chemical process shall be solely owned by Supplier. All of the intellectual property on or related to the End Product or components thereof, except the API, shall belong to the Buyer. Supplier shall neither use nor transfer to a third party its knowledge of Buyer's End Product; including, but not related to its chemical make-up, its formulation or any other of its physical specifications all of which shall be considered as Buyer's Confidential Information.

16.2 The intellectual property rights and know-how related to the manufacture of the API shall remain with Supplier. For the avoidance of doubt this refers to the equipment, methodology, engineering, research techniques etc. owned by Supplier as of the Effective Date, and that have been deployed and used by Supplier during the research provided for the formulation of the End Product and its subsequent scale-up and industrialization and that can be deployed by Supplier for other Supplier clients for different products

16.3 Notwithstanding the foregoing, both Parties shall retain full right and title to any intellectual property they may have developed prior to the Effective Date of this Agreement or without any reference to information received from the other Party under this Agreement. All intellectual property developed during the course of this Agreement or the manufacture of the End Product, excluding the API, shall belong to the Buyer; and shall be promptly assigned and transferred to the Buyer, and the Supplier hereby agree to transfer and assign to the Buyer, without any additional consideration, fee or charge.

16.4 In the event that a third party claims ownership to the intellectual property of the API, Supplier shall notify Buyer and Supplier shall comply with Buyer's instructions to defend, settle or litigate such claim. Supplier shall indemnify, defend and hold Buyer harmless from and against any such third party API claim related to Supplier's ownership and use of the intellectual property rights in the API

17. Confidentiality

17.1 Both Parties shall keep, now and at all times in the future, all information exchanged in relation to the API and the End Product and this Agreement In strict confidence, specifically all technical information related to the API and the End Product's physical specifications, manufacturing process, regulatory information as well as any industrialization, engineering or technical information that either Party may make the other Party aware of.

17.2 In the event of doubt, both Parties shall consider information received from the other Party as confidential regardless whether such information was provided verbally, in written form, regardless of support or if obtained by accessory during a Site visit etc.

17.3 Either Party shall notify the other Party of any suspected breach or any court order or competent authority request to obtain such information.

17.4 Either Party shall ensure that adequate safeguards are in place to restrict access to such confidential information to the personnel required to have access in order to perform such Party's obligations under this Agreement or any other specific arrangement (such as a research arrangement).

17.5 Any information that is: (i) already in the public domain or (ii) lawfully obtained from a third party shall not be considered as confidential.

17.6 In the event of a court order, or request from a competent authority, for information received from the other Party, then the Party receiving such order or request shall inform, to the extent possible the other Party and cooperate fully with such other Party, to the extent permitted under law, for the safeguard of such information's confidentiality.

18. Intentionally Deleted

19. Force Majeure

19.1 Neither Party shall be in breach of this Agreement nor liable for delay or failure in performing any of its obligations under this Agreement if such delay or failure result from any circumstances beyond the reasonable control of the party affected, including by way of example, acts of God, acts of terrorism, fires, floods, wars, sabotage, accidents, labour disputes or shortages, plant shutdown, equipment failure, machine breakage, compliance with any law, order, rule or regulation of government agency or authority, or inability to obtain materials and utilities (including power and fuel), equipment or transportation ("**Force Majeure Event**").

19.2 Upon occurrence of a Force Majeure Event, the affected Party shall immediately notify the non-affected Party and shall keep the non-affected Party appraised of the situation.

19.3 This Agreement shall be suspended for the duration of the Force Majeure Event to the extent that a Party is unable to carry out its obligations (e.g. if a Force Majeure Event creates unavoidable delays then Supplier will only be released from its obligation to meet the delivery date but not from its obligation to manufacture and supply the API under the affected Purchase Order). Both Parties shall cooperate with each other to mitigate the effects of a Force Majeure Event on the other Party.

20. Representations and Warranty

20.1 Supplier represents and warrants that the API will be manufactured and delivered in accordance with cGMP regulations and shall conform to all USP Specifications, ICH guidelines, US FDA DMF requirements and this Agreement.

20.2 Supplier further represents and warrants that the API will be labelled and packaged in accordance with Buyer Specifications and cGMP rules.

20.3 Supplier further represents and warrants that the API will be manufactured in accordance with the Specifications and this Agreement.

20.4 Supplier provides no further warranty or assurance than as expressly specified in this section and, except as noted herein, provides no warranty as to the specific performance of the End Product except that it will perform in accordance with all Specifications.

20.5 Supplier represents and warrants that it has all required permits and licenses to operate its Facilities at the Site and to manufacture the API, and to enter into this Agreement.

20.6 Each Party hereby represents and warrants to the other as of the Effective Date that:

(a) It is a duly organized and validly existing corporation, and is in good standing under the laws of the jurisdiction of its organization, and has all requisite power and authority, corporate or otherwise, to execute, deliver, and perform this Agreement.

(b) This Agreement is a legal and valid obligation binding upon such Party and enforceable in accordance with its terms, subject to the effects of bankruptcy, insolvency, or other laws of general application affecting the enforcement of creditor rights, judicial principles affecting the availability of specific performance, and general principles of equity (whether enforceability is considered a proceeding at law or equity),

(c) The execution, delivery, and performance of this Agreement by such party does not conflict with any contract or understanding to which such Party is a party or by which it is bound.

(d) The execution, delivery, and performance of this Agreement by such Party have been duly authorized by all necessary action, corporate or otherwise, and do not violate any applicable law or any order, writ, judgment, injunction, decree, determination, or award of any court or governmental body, or administrative or other agency presently in effect applicable to such Party.

(e) It is not aware of any government authorization, consent, approval, license, exemption of or filing or registration with any court or governmental department, commission, board, bureau, agency or instrumentality, domestic or foreign, under any applicable law, currently in effect, necessary for, or in connection with, the transactions contemplated by this Agreement or any other agreement or instrument executed in connection herewith, or for the performance by it of its obligations under this Agreement and such other agreements.

(f) No Party is under any obligation, contractual or otherwise, to any person that conflicts with or is inconsistent in any material respect with the terms of this Agreement, or that would impede the diligent and complete fulfillment of its obligations hereunder.

21. Insurance and records

21.1 **Insurance**: Both Parties represent and warrant that they will carry adequate Insurance for the purpose of performing their obligations under this Agreement and shall provide, upon request, proof of such insurance.

21.2 **Records**: Supplier shall keep full records of the API, including related raw materials, manufacture, testing, storage etc. for a period of [***] following the production of each API Purchase Order.

22. Limitation of Liability

22.1 **Cap of Liability**. Except in cases of [***], Supplier's total liability in contract, tort (Including negligence) or otherwise in relation to this Agreement will be limited to [***].

22.2 **Exclusion of certain liabilities**. In any event, even if Supplier is advised in advance of the possibility of any loss or damage, Seller shall not be liable to the Purchaser for:

[***]

23. Notifications

23.1 All notifications shall be in writing and shall be made by registered or certified letter to the address listed in the above with a copy by email to the persons listed below for each respective Party. Delivery in person is also permitted and satisfies the conditions of this Agreement.

23.2 For Supplier the notified persons are: (Sales) [***] and (Legal Director) [***]

23.3 For Buyer the notified persons are: [***] and (Legal) [***].

23.4 In the event of a change in one of the person's listed above, the Party affected by such change shall inform the other Party.

23.5 When a Party responds to a notification or otherwise acknowledges receipt, even if irregular under this Agreement, then such notification shall be considered as valid.

24. Term and termination

24.1 This Agreement shall be effective as of the Effective Date and shall remain in full force and effect for an initial period ending five (5) years after the Effective Date.

24.2 Upon expiry of such initial period, the Agreement shall be automatically renewed for subsequent renewal periods of two (2) years each.

24.3 Either Party may terminate this Agreement at the end of the initial period or a subsequent renewal period without liability upon prior written notification sent at least [***] in advance.

24.4 Either Party may further terminate this Agreement in the event that:

24.4.1 The other Party has committed a material breach of any of its obligations under this Agreement and such breach has not been remedied within [***] of written notification of such breach by the offended Party;

24.4.2 The other Party enters into insolvency proceedings or any related restructuring or bankruptcy proceedings;

24.5 Effects of termination: In the event of termination by either Party, the following shall occur upon termination:

24.5.1 Supplier shall manufacture and Buyer shall take delivery of and pay for all Purchase Orders placed prior to termination in accordance with this Agreement; and

24.5.2 Supplier shall transfer to Buyer all Buyer intellectual property or to its designated contractor It being understood that if this requires significant resources such time may be recharged to Buyer;

25. Jurisdiction and governing law

25.1 This Agreement shall be governed exclusively by the laws of England and Wales.

25.2 All disputes arising out of or in connection with the present contract shall be finally settled under the Commercial Rules of Arbitration (the "**Rules**") of the International Chamber of Commerce ("**ICC**") in Geneva, Switzerland, by one (1) arbitrator appointed in accordance with the said Rules.

25.3 This Agreement is made in English language and shall be interpreted in English language.

26. Miscellaneous

26.1 **Survival**: all clauses related to intellectual property, confidential information, governing law and jurisdiction shall survive termination or expiration of this Agreement and remain in force for [***] thereafter.

26.2 **Entire Agreement**: This Agreement, together with its Exhibits and subsequent Purchase Orders or documents issued in its performance, constitutes the entire understanding between the Parties in the subject matter thereof. In the event of a conflict between its terms, this Agreement shall supersede its Exhibits and together they shall supersede any Purchase Orders. By exception the Quality Agreement shall take precedence for quality issues. Each Party agrees that it has not relied on any representation, warranty, collateral contract or other assurance (except those expressly set out in this Agreement) made by or on behalf of the other party before the signature of this Agreement. Each party waives all rights and remedies which, but for this present clause, might otherwise be available to it in respect of any such representation, warranty, collateral contract or other assurance

26.3 **Severability**: If any provision of this Agreement is found by a court of competent jurisdiction to be invalid or unenforceable, the invalid or unenforceable part or provision shall be replaced with a provision which accomplishes, to the extent possible, the original business purpose of such part or provision in a valid and enforceable manner, and the remainder of this Agreement shall remain binding upon the Parties hereto.

26.4 **Change Control**: neither Party may terminate this Agreement in the event of a change In control of the other Party unless such change results in the other Party being controlled by, controlling, or being under common control with a direct competitor of the other Party.

26.5 **Assignment**: Neither Party may assign its rights nor its obligations under this Agreement to a third party without the other Party's prior written consent; provided however, that in the event of the sale of all or substantially all of the securities, assets or business of Buyer to which this Agreement relates, or the merger business acquisition or other combination of the Buyer with a third party, this Agreement shall be automatically assigned and transferred to the Buyer's successor without the required consent or approval of Supplier so long as the assignee assumes in writing all of the terms and conditions of this Agreement.

26.6 **Amendment**: This Agreement may only be amended by written form executed by both Parties. It is understood, that minor amendments, changes to the Exhibits or the notified persons' details and changes to Purchase Orders may occur through exchange of email. Failure by a Party to exercise a right for a period of [***] following the event giving rise to such right shall constitute a waiver of such right.

26.7 **Independent contractors**: The Parties are acting as an independent contractor. This Agreement may not be construed as creating or constituting a partnership, joint venture, or agency relationship between the Parties. Neither Party will have the power to bind the other or incur obligations on the other's behalf without the other's prior written consent.

26.8 **Third Party rights**: Any person not Party to this Agreement shall not have any right to enforce any term of this Agreement.

26.9 **Counterparts**: This Agreement may be executed in several counterparts in original copy or by electronic copy. A scanned executed version sent by either Party shall be considered as executed by such Party as an original copy.

SIGNATURE PAGE FOLLOWS

IN WITNESS WHEREOF, the Parties hereto have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

For Supplier
Name: Frederic Desdouits

Title: CEO

Signature: /s/ Frederic Desdouits

For Buyer Amylyx Pharmaceuticals, Inc.
Name: Justin Klee

Title: President

Signature: /s/ Justin Klee

List of Exhibits:

- Exhibit 1: Specifications
- Exhibit 2: Price
- Exhibit 3: Quality Agreement
- Exhibit 4: Form of Purchase Order

Exhibit 1: Specifications

PRODUCT SPECIFICATION

PRODUCT:	[***]	
Trade Name:	[***]	
CA-Registry-No.:	[***]	[***]
Date of issue:	[***]	

SPECIFICATION:

Appearance:	[***]
Identify:	[***]
Assay:	[***]
Impurities:	[***]
Water Content	[***]
Heavy	[***]
pH-Value (2%, aqueous solution)	[***]
Residual Solvents:	[***]

The information submitted in this publication is based on our current knowledge and experience. It does not relieve the purchaser from examining the product upon delivery and gives no assurance of suitability of the product for any particular purposes

CU Chemie Uetikon GmbH
Raiffeisenstrasse 4 - D-77933 Lahr – Germany
Tel.: (*+49)(7821)585 208 - Fax.: (++49)(7821)585 0
E-Mail; info@uetikon.com - http://www.uetikon.com

Exhibit 2: Price

The price of the Product, inclusive of packaging and to the agreed INCOTERM, will depend upon the quantity of Product ordered by the Buyer for delivery during each calendar year, as shown in the table below:

Quantity Range (1)	***Price €/kg (2)***
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

Minimum order quantities are [***]

Where the aggregate quantities of Product ordered by the Buyer for delivery in a calendar year are as specified in Column (1) above, and the price applicable for all of such Product ordered for delivery in that calendar year will be as specified opposite that quantity in Column (2) above.

Price Reconciliation

During each calendar year, Supplier will invoice the Buyer at a price which assumes that during that calendar year, the aggregate quantity of Product which the Buyer will order will be as specified in the Forecast applicable to that calendar year.

By the end of January in each calendar year, Supplier will calculate the aggregate quantity of Product actually ordered by the Buyer for delivery in the previous calendar year, and will compare this to the quantities specified in the Forecast relevant to that calendar year.

Supplier will either invoice the Buyer, or issue a credit note, (as applicable) for the sum necessary to result in a position where the price actually paid by Buyer for Product ordered for delivery during the previous calendar year is in accordance with the price structure set out in above.

Exhibit 3: Quality Agreement

[***]

Exhibit 4: Form of Purchase Order

43 Thorndike Street
Cambridge, MA 02141
617-5 71 -88 72

[***]

Purchase Order	Amylyx Pharmaceuticals Inc. 43 Thorndike Street Cambridge, MA 02141

P.O. Number: XXXXXX

P.O. Date: XXXXXX

Description	Quantity X Kilos	Price

Purchase Order Total:

Please Ship to:

Please Invoice Amylyx Pharmaceuticals, Inc.

Amylyx Pharmaceuticals, Inc.
43 Thorndike Street
Cambridge MA 02141

Amylyx EIN: 46-4600503
Contact Phone
[***]

Authorized Signature:

Amylyx Pharmaceuticals, Inc.

Joshua Cohen, CEO

Certain identified information has been excluded from this exhibit because it is both not material and is the type that the registrant treats as private or confidential. Information that was omitted has been noted in this document with a placeholder identified by the mark "[***]".

RESEARCH, DEVELOPMENT AND SUPPLY AGREEMENT

This agreement (the "**Agreement**") is made and entered into as of December 9th, 2019 (the "**Effective Date**")

by and between

Prodotti Chimici e Alimentari S.p.A., a corporation duly organized and existing under the laws of Italy with a place of business at Via Novi 78, 15060, Basaluzzo (AL), Italy as represented by Paolo Oligeri in his capacity of CEO (hereinafter also referred to together with its Affiliates (as defined below) as "**PCA**")

And

AMYLYX PHARMACEUTICALS, INC., a corporation duly organized and existing under the laws of The State of Delaware with a usual place of business at 43 Thorndike St, Cambridge, MA 02141 as represented by Thomas Holmes in his capacity as Global Head, Supply Chain (hereinafter also referred to together with its Affiliates (as defined below) as "**AMYLYX**")

(PCA and AMYLYX being also hereinafter referred to individually as "**Party**" and jointly as "**Parties**"),

whereas:

(i) PCA has experience in the development and manufacture of active pharmaceutical ingredients, including Taurursodiol [***], for use by third parties in finished pharmaceuticals products;

(ii) AMYLYX is a pharmaceutical company involved in the development, manufacturing and marketing of medicinal specialties;

(iii) PCA has developed Taurursodiol [***] (hereinafter "**API**"), and [***], specifically for AMYLYX, carrying out significant chemical and technical searches, bearing the related costs, for the development of the API (hereinafter, "**Research & Development Activities**");

(iv) PCA is now completing the said researches, manufacturing and supplying the API to AMYLYX with the aim of going through the clinical phases and obtaining approvals for pharmaceutical use, and, to this end, PCA has carried out and is carrying out regulatory and formal accomplishments dealing with the relevant offices and authorities aimed at obtaining the necessary permits, approvals and/or authorization to manufacture and supply the API, bearing significant costs (hereinafter, "**Regulatory Activities**");

(v) AMYLYX now desires to produce and commercialize throughout the world (hereinafter the "**Territory**") a Pharmaceutical Product for the treatment of the rare disease known as Amyotrophic Lateral Sclerosis (hereinafter "**Finished Product**" as defined below), after AMYLYX obtains the regulatory approvals from the applicable Competent Regulatory Authorities in the Territory to use the API manufactured by PCA;

(vi) The Commercial Launch (as defined under Section 1.2 below) by AMYLYX of the Finished Product is anticipated and planned between [***] to [***] subject to approval of all Regulatory Activities;

(vii) AMYLYX, therefore, is willing to purchase from PCA the quantity of API which might be necessary to produce the Finished Product; and PCA is willing and agrees to continue the manufacture and supply the API to AMYLYX throughout the Territory, as per the terms and conditions set forth in this agreement and in the Exhibits attached hereto.

NOW THEREFORE, in consideration of the foregoing and for other good and valuable consideration, including the herein agreements, covenants, promises, representations and warranties, the receipt and legal sufficiency of which is hereby acknowledged, accepted and agreed to by the Parties, and intending to be legally bound, the Parties hereby agree as follows:

1. Preliminary matters

1.1 **Contractual documents**. The preamble and the Exhibits listed below shall constitute an integral and substantial part of this agreement (hereinafter referred to as "**Agreement**"):

Exhibit "A": Unit price
Exhibit "B": Specifications of the API (the "**Specifications**")
Exhibit "C": Commercial Launch Plan

1.2 **Definitions**. To the extent of this Agreement, abbreviations and expressions shall have the meaning reported below:

Additional Period: the period of two (2) years following the three (3) years period after the Commercial Launch.

Affiliate: shall mean, with respect to any Party, any natural person, joint venture, partnership, corporation, trust, unincorporated organization or other entity ("**Person**") which, directly or indirectly, controls, is controlled by, or is under common control with, such Party. "**Control**" shall mean the ownership of 50% or more of the issued share capital or the legal power to direct or cause the direction of the general management and policies of the Person in question.

API: shall mean the active pharmaceutical ingredient Taurursodiol [***].

Batch: shall mean a defined quantity of bulk API, which is manufactured together in one process as established in the DMF.

Certificate of Analysis: shall mean the certificate that accompanies each Batch of API and which lists the test methods, acceptance limits and release test results of that specific Batch.

cGMPs: shall mean the good manufacturing practices (GMP) applicable at the time of the manufacturing in the European Union ("**EU**"), the US, and/or in the other countries of the TERRITORY in relation to the production of drugs.

Commercial Launch: shall mean the date on which AMYLYX begins to distribute the Finished Product to third party customers in the ordinary course of its business.

Competent Regulatory Authority: shall mean any local, national or supranational agency, authority, department, ministry, or official of any government of any country having jurisdiction over this Agreement or any of the Parties hereto, or over the development, production or marketing of the API or Finished Product.

Confidential Information: All information of whatsoever nature (whether oral, written, electronic or in any other form) including without limitation: data, know-how, trade secrets, manufacturing processes and systems, open part of the Drug Master File (Customer Profile), registration dossiers, scientific and/or clinical data, formula and formulations, samples of goods, software techniques, procedures, test methods, unpublished financial statements and information, licenses, prices, price lists, pricing policies, customer and supplier lists, customer and supplier names and other information relating to customers and suppliers, marketing techniques and marketing development tactics and plans, and all other information containing or consisting of material of a technical, operational, administrative, economic, marketing, planning, business or financial nature or in the nature of Intellectual Property disclosed by one Party to the other Party.

Contract Year: means each consecutive 365-day period during the Term, the first of which commences on the Effective Date.

Defect: shall mean any instance where the API fails to conform to the Specifications or to the provisions of Section 8.1, as must be duly assessed and analysed by AMYLYX or its designee/s following receipt of the API.

Defective API: shall mean the API that does not comply with the Specifications or the provisions of Section 8.1.

DMF: shall mean the Drug Master File for the API and any other documents serving a similar purpose filed with the FDA or any other Competent Regulatory Authority.

FDA: shall mean the United States Federal Food and Drug Administration and any successor agency having substantially the same function as well as any similar regulatory agency in the Territory.

Finished Product: shall mean a Pharmaceutical Product incorporating the API or produced using the API as a precursor or ingredient for the treatment of Amyotrophic Lateral Sclerosis.

Intellectual Property: shall mean any patent, copyright, database right, design right, registered design, registered or *de facto* trademark, service mark, domain name, know-how, trade secrets, utility model, unregistered design and any application for any of the foregoing or any improvements, enhancements, discoveries or inventions of any of the same, or other industrial or intellectual property right.

Law: shall mean all applicable supranational, state, local or foreign statute or law and shall be deemed also to refer to all rules and regulations promulgated there under by the Competent Regulatory Authorities, unless context requires otherwise. Any reference to a particular law or regulation will be interpreted to include any revision of or successor to such statute, law, rule or regulation regardless of how it is numbered or classified.

NDA: shall mean the New Drug Application filed with the FDA by AMYLYX with a view to obtaining the approval(s) required under Law for producing and commercializing the Finished Product.

Territory: shall mean the World.

Pharmaceutical Product: shall mean any product, regardless of whether used for research, clinical trial, samples or commercial purposes and regardless of where actually or proposed to be marketed or sold, which, if such product were marketed or sold in the United States, would be considered a "drug" (whether prescription, generic or over the counter) under the United States Federal Food, Drug and Cosmetic Act, as may be amended from time to time and any regulations there under.

Price: the sum set forth in Exhibit "A" that AMYLYX shall pay to PCA for the delivered API.

Regulatory Activities: shall mean the regulatory and formal accomplishments that PCA, dealing with the relevant offices and authorities, carried out in order to obtain the necessary permits, approvals and/or authorization to manufacture and supply the API.

Research & Development Activities: shall mean the chemical and technical searches that PCA carried out to develop the API specifically for AMYLYX.

Specifications: shall mean the written specifications and quality standards, including tests, analytical procedures and acceptance criteria established by PCA to confirm the characteristics and quality of API as specified under Exhibit B.

1.3 **Interpretation of the Agreement**. This Agreement, together with all Exhibits, constitutes the entire agreement between the Parties with respect to the subject matter hereof and shall supersede and cancel all prior understandings, whether oral or written. Any change to this Agreement and/or any Exhibits, will be valid solely if agreed upon in writing by the Parties.

Any provision herein which may in any way contravene the Law shall be deemed, to the extent of such contravention, severable and of no force and shall not affect any other provision of the Agreement.

The English language version of this Agreement executed by the Parties is the original and sole, final and binding version of this Agreement and shall control over any translation thereof into any other language.

2. Undertakings related to the Research and Development and Regulatory Activities and to the launch of the Finished Product

2.1 **PCA undertakings**. Without prejudice to provisions under Article 8 below, PCA shall be responsible for the Research and Development and Regulatory Activities, including prompt preparation submission and updates of a DMF for the API, and all costs and fees associated with such activities, including the correct and timely payment of site registration fees and DMF filing fees. PCA undertakes to manufacture and supply the API pursuant to Article 3 of this Agreement.

2.2 **AMYLYX undertakings**. AMYLYX shall be responsible: (i) for the filing of the NDA and of any other- drug applications required by the Competent Regulatory Authority and/or by Law for producing and commercializing the Finished Product in each relevant country within the Territory, and for the maintenance and all activities related to such NDA and other drug applications; (ii) for the manufacture, marketing, sale, labeling and promotion of the Finished Product and (iii) for the compliance with the Law applicable to the manufacture and marketing of the Finished Product. Recognizing that the Commercial Launch of the Finished Product is subject to prior FDA's approval of the NDA, AMYLYX shall use its commercially reasonable efforts, at its sole expenses, to launch the Finished Product by the expected date of Commercial Launch as indicated in paragraph (vi) of the preamble and pursuant to the time schedule as set out in Exhibit C, which forms an integral part of this Agreement ("**Commercial Launch Plan**").

2.3 **Cooperation**. During the term of this Agreement, PCA shall assist and cooperate in a timely manner with AMYLYX in its preparation of any documents or other materials, which may be required by the FDA and/or any other regulatory authority to validate, sell, and/or distribute the API to be supplied by PCA under this Agreement or the Finished Product. PCA shall file with the FDA and/or any other Competent Regulatory Authorities, and shall maintain at all times as current, a DMF for the API.

3. Supply of API

3.1 **Requirements**. Subject to the terms of this Agreement, AMYLYX hereby agrees to purchase from PCA during the term of this Agreement [***] for the research, development and commercialization of the Finished Product and PCA hereby agrees to manufacture and supply to AMYLYX, in accordance with the terms of this Agreement [***], for the development and commercialization of the Finished Product. PCA, in addition, and without any additional; charge, fee or cost, grants to Amylyx, the right, to refer to and to use for the sole purpose of manufacturing and commercializing the Finished Product and subject to the Term of this Agreement, the DMF relating to the API necessary for the registration of and regulatory submissions with respect to the Finished Product. Notwithstanding anything to the contrary herein contained, PCA represents and warrants that it will have and it will continue to have during the "**Launch Period**", as hereinafter defined, the ability, capacity and materials to timely manufacture and supply to AMYLYX, and agrees to provide to AMYLYX, all API ordered by AMYLYX in accordance with the Forecast as defined and provided in Section 4 below and, in particular, without limitations, being fully applicable the procedures therewith provided under Section 4.1(a) and 4.1(b). For purposes of this Agreement, the Launch Period is defined to mean the [***] period commencing on the Effective Date and terminating at the end of the [***] following the Effective Date.

3.2 AMYLYX's commitment referred under Section 3.1 shall remain in effect so long as PCA is able to fulfill AMYLYX's requirements for quality, quantity and timely delivery of the API, pursuant to the provisions set forth herein. Otherwise, AMYLYX shall be entitled to purchase all portions of needed API from an alternative qualified supplier, as per Section 13.6 below without any breach or violation of this Agreement.

AMYLYX acknowledges that the API shall be used solely for the purpose of developing, producing and commercializing the Finished Product in accordance with all Regulatory Activities and in compliance with Law.

4. Forecast

4.1 **Forecast**.

(a) In order to enable PCA to plan the manufacturing process of the API, AMYLYX shall, within [***] days of the Effective Date, and then subsequently within [***] days of the commencement of each calendar quarter, transmit a forecast of its requirements of API and of estimated delivery dates for the following twelve (12) month period ("rolling twelve (12) month forecast"). Within [***] days of receiving AMYLYX's rolling twelve (12) month forecast, and promptly at any other time that PCA identifies or reasonably anticipates a material problem that impairs its ability to supply AMYLYX, PCA will communicate to AMYLYX any prospective problems it might have in respect to meeting forecasted quantities or estimated delivery dates and both Parties shall use their commercially reasonable efforts to remove the problems and/or to schedule otherwise the quantities and/or the delivery dates.

(b) In order to assist AMYLYX to understand the impact of AMYLYX's purchases of API on PCA's production and capacity, PCA and AMYLYX shall meet in-person at least [***] per year and at least [***] by conference call and more regularly as agreed to by the Parties, to evaluate AMYLYX's planned purchases of API and PCA's abilities to meet AMYLYX's planned demand.

4.2 **Firm and indicative forecast**. The first [***] of each rolling twelve (12) month forecast shall be firm and binding for both Parties. Thus, except as provided for by Section 4.1 (a) above, PCA agrees to supply one hundred percent (100%) of the quantities ordered by AMYLYX for such a period.

The remaining period of each rolling twelve (12) month forecast (and the related quantities) shall be indicative only and not binding on either Party, provided that the quantities actually ordered by means of issuing purchase orders may deviate, upwards or downwards, from the most recent non-binding rolling twelve (12) month forecast to no more than [***] or less than [***] respectively. Thus, PCA agrees to use its commercially reasonable efforts to allocate manufacturing capacity and keep stock of raw materials and API sufficient to meet the non-binding portions of the then-current rolling twelve (12) month forecast and to accommodate any further increases in the quantity of API that AMYLYX shall request under new purchase orders.

AMYLYX agrees to purchase the inventory on hand of API manufactured by PCA in order to meet the [***] quantity forecasted by AMYLYX and which has not yet been purchased by the same at termination of this Agreement as per Article 13 below.

5. **Purchase orders**

5.1 Commencing on the date on which AMYLYX receives the relevant regulatory approval to sell the Finished Product, including the API manufactured by PCA, for commercial use in the Territory, AMYLYX will purchase the API under this Agreement by submitting written purchase orders ("**P.O.**") [***] days prior to each delivery date as estimated in the then-current rolling twelve (12) month forecast. Each P.O. shall set forth: (i) the quantity of API; and (ii) the delivery address and INCOTERMS agreed upon in advance between the Parties.

5.2 The Parties agree that this Agreement, as amended from time to time, governs the commercial terms between them with respect to AMYLYX's purchase of the API from PCA. Therefore, any terms or conditions set forth in a P.O. (other than those specified to be included by the preceding paragraph) that are either inconsistent with or in addition to the terms and conditions of this Agreement shall not be binding on either Party and shall have no force or effect.

5.3 PCA shall accept in writing each P.O. promptly upon its receipt, unless the quantity specified in such P.O. is not in accordance with the terms of this Agreement, in which case PCA shall state in writing its reasons for rejecting any such P.O. as submitted by AMYLYX. In no case shall a P.O. be considered as accepted in the absence of a written confirmation from PCA as above. Any terms and conditions in PCA's order acceptance that are either inconsistent with or in addition to the terms and conditions of this Agreement shall not be binding on either Party and shall have no force or effect.

5.4 Each P.O. issued and accepted as per 4.1 above is to be deemed as binding for both Parties, without prejudice to any other provision set forth herein or in a subsequent agreement in writing between the Parties.

6. **Delivery and acceptance**

6.1 **Delivery**. The delivery of the API to AMYLYX shall be carried out "CIP/EX WORKS" the ship-to location specified, pursuant to Section 5.1 above, in the purchase orders issued by AMYLYX to PCA, as such term is defined in ICC INCOTERMS (2010).

The API shall be delivered in PCA's normal packaging, as substantially reflected in its DMF and/or in the Specifications attached hereto.

PCA shall use its commercially reasonable efforts to deliver the API by the estimated delivery date. PCA shall notify AMYLYX in writing if PCA has reason to believe that the actual delivery will not occur within [***] days of the estimated delivery date. In the event that PCA cannot deliver API to AMYLYX within [***] days after the delivery date specified in a P.O., PCA agrees that AMYLYX may, in its sole discretion, elect to cancel such portion of a P.O. relating to such API and procure the API pursuant to Section 13.6, or accept partial or complete delivery at a later date specified by PCA.

6.2 **Inspection and acceptance**. The delivered API shall be deemed as fully accepted if AMYLYX does not notify PCA of the API's proved Defect within [***] days after the documented date of receipt unless the Defect is a latent Defect that could not reasonably be discovered within [***] days in which event, AMYLYX shall notify PCA within [***] days of discovery. Thus, AMYLYX shall endeavour to promptly inspect the API upon receipt, at its own cost, pursuant to the cGMPs regarding the controls to be performed by the manufacturer of a Pharmaceutical Product over raw and incoming materials and shall give—by the aforementioned term — written notice of any claim, setting forth the details and giving evidence of such Defect or a latent Defect as the case may be.

6.3 **Disputes**. The Parties agree to consult with each other to resolve the discrepancy between each other's determinations, if any, about any alleged Defect. If such consultation does not resolve the discrepancy within [***] days after identification of the discrepancy, then the Parties shall nominate a mutually agreeable reputable independent laboratory to test representative samples taken from such shipment. The results of such tests shall be binding on the Parties and the expense of such tests shall be borne by the Party against which the laboratory decided.

6.4 **Reimbursement or Replacement**. Subject to the resolution of any disputes regarding the Defective API pursuant to Section 6.3, if AMYLYX has paid for the Defective API, then PCA shall at no additional cost, using commercially reasonable efforts, promptly cause such Defective API to be replaced with API conforming to provisions under Section 8.1 or, at AMYLYX's option, credit AMYLYX for the amount paid for such Defective API. All Defective API shall upon the request of PCA, and at its sole cost, be returned to PCA at the address set forth herein, packed and shipped according to instructions provided by PCA at PCA's expense.

6.5 In case of Defective API, AMYLYX—if necessary—may utilize a secondary supplier selected pursuant to Section 13.6 as reasonably necessary to fulfil its requirements. AMYLYX may only make use of such secondary supplier in the event PCA is unable to fulfil any of its obligations as provided under Section 13.4 of this Agreement.

6.6 In case the Defect would be due to improper handling or storage conditions subsequent to delivery of the API, no liability will occur to PCA.

7. **Price and payment**

7.1 **Price**. AMYLYX shall pay to PCA for the delivered and accepted (which acceptance shall occur within [***] days of delivery), API the sum set forth in Exhibit A. including all applicable taxes, fees and export duties, according to the agreed Incoterms (hereinafter, "**Price**"). The Parties acknowledge and agree that the Price is fair and has been negotiated in good faith also taking into account: (i) the Research & Development and Regulatory Activities carried out and to be carried out by PCA; (ii) the related costs incurred by PCA and those that PCA shall bear in the performance of this Agreement with reference to the API; (iii) the expected date of Commercial Launch as represented by AMYLYX to PCA and the consequent volumes of API to be supplied by PCA.

7.2 The Price shall remain valid and in effect for [***] years as of the Commercial Launch of the Finished Product. After such [***] years period, the Price shall automatically further apply for an additional period of [***] years (hereinafter "**Additional Period**"), unless the Parties mutually agree upon a new price pursuant to the following Section 7.3.

7.3 In any event, upon completion of the Additional Period, if applicable, the Parties shall mutually agree to decrease, maintain or increase the Price with good faith negotiations to start [***] months prior to the above-mentioned term. The new Price agreed shall remain valid and in effect for the term agreed between the Parties or, in the absence of any such agreement, for [***] and will be indicated in Exhibit "A", by means of a new Exhibit "A", that will be attached to this Agreement.

7.4 In the event the Parties do not reach an agreement within [***] months from the commencing of the negotiations period referred to in Section 7.3, either Party shall have the right to terminate this Agreement by means of [***] months prior written notice to the other Party, it being understood that during such notice period the last Price agreed to by and between the Parties shall apply and be in effect.

7.5 **Invoicing and payment**. PCA shall submit the invoice to AMYLYX upon shipment of the API and the full invoiced amount shall be paid by AMYLYX within net [***] days after the receipt of the invoice? All payments hereunder shall be payable in USD.

8. **Quality and recall of the API**

8.1 **Quality**. PCA warrants that the API will be of standard quality and will be manufactured in compliance with Law and cGMPs, as per the Specifications and the DMF. API delivered shall have a minimum retest date of not less than [***] years).

Quality of the API shall be according to the mutually agreed Specifications, as well as any official standards in the Territory applicable at the Effective Date, as detailed and confirmed by the DMF. Therefore, should new, more restrictive specification(s) become effective through new compendial monographs during the validity of this Agreement, the Parties will mutually evaluate in good faith the impact of the same on the performance of the Agreement.

8.2 **Certificate of Analysis**. When each Batch is delivered API shall be accompanied by a Certificate of Analysis stating the compliance of the API as per above. AMYLYX acknowledges, however, that it shall not be entitled to rely on the Certificate of Analysis or any such other certificate without the necessity for performing additional tests.

8.3 **Recall**. Except as herein noted, PCA shall not be responsible for the costs of any recall of the Finished Product ordered by a Competent Regulatory Authority or any other government agency or tribunal. However, if AMYLYX proves to PCA's reasonable satisfaction, or a Competent Regulatory Authority or any other government agency or tribunal determines that the need for the recall arose, in whole or in part, from a demonstrated latent Defect in the API not detectable by AMYLYX during the inspections to be performed by the manufacturer of a Pharmaceutical Product over the incoming active ingredients and materials pursuant to Laws and cGMPs and pursuant to provisions under Section 6.2, AMYLYX shall notify PCA in writing within [***] days after the relevant Defect has come to AMYLYX's notice and not more than [***] after the receipt of the Finished Product. If the Parties disagree as to whether such recall arose, in whole or in part, from a demonstrated Defect in the API, the matter will be submitted

to a mutually agreeable reputable independent laboratory for analysis to determine whether the API conformed or did not conform to the Specifications and/or requirements of Section 8.1. The test results obtained from such laboratory shall be final and binding upon both Parties. The fees and expenses of such testing shall be borne by the Party ultimately determined to have incorrectly judged whether the API met the said requirements. In the event that the API is deemed defective, PCA shall reimburse AMYLYX for all or a portion (in accordance with the following Section 8.4) of the reasonable and documented costs incurred in order to proceed such recall activities, or otherwise to comply with the order of the Competent Regulatory Authority or any other government agency or tribunal.

8.4 The Parties agree that the portion of the recall costs to be reimbursed by PCA under Section 8.3 shall be proportional to the relative role of such demonstrated defect in the API in causing the recall and the costs of recall activities..

8.5 **Regulatory Registration**. In so far as is necessary to allow for the production of the API and its importation and sale into the Territory, PCA will register its manufacturing facilities and the API with the FDA and any appropriate Competent Regulatory Authority, maintain at its sole cost said registrations during the term of this Agreement, allow inspections of such facilities by the FDA and foreign governmental authorities, maintain and make available to the FDA and other governmental authorities records, files, and any other documents relating to the manufacture, storage, shipping and quality of the API. PCA further agrees to work with AMYLYX to enhance the DMF as reasonably suggested by AMYLYX, or by the FDA or other Competent Regulatory Authority, including, without limitation, providing AMYLYX access to PCA personnel responsible for production, testing and validation and any related documentation, information or materials, so as to enable AMYLYX to obtain timely approval from the FDA or other Competent Regulatory Authority for the utilization of the API in the manufacture, distribution and sale of the Finished Product formulated using API manufactured by PCA. PCA further grants to AMYLYX and its Affiliates the right to refer to and to use for the sole purpose of manufacturing and commercializing the Finished Product and subject to the Term of this Agreement, the DMF relating to the API necessary for the registration of and regulatory submissions with respect to the Finished Product. PCA represents and warrants that it shall, at all times, maintain and keep current the DMF and that it shall fully cooperate and timely respond to any inquiry, comment or deficiency notification from the FDA or any other Regulatory Authority as to the DMF.

9. **Records, audit and information**

9.1 **Records**. PCA will keep complete and accurate records regarding the manufacture and the supply of the API, as required by Law, and agrees to give information regarding such records to AMYLYX, provided that the transmission of such information or records is permitted by applicable Law and is required by AMYLYX in connection with any FDA inspection or in order to obtain or maintain the approvals for the Finished Product from Competent Regulatory Authorities. PCA will provide AMYLYX full access to the open part of the DMF for the API.

9.2 **Audit**. Upon receipt of [***] days prior written notice from AMYLYX, PCA shall allow inspection of those areas of the facilities during normal business hours where the API is manufactured for AMYLYX or Affiliates. Upon receipt of [***] days written notice from AMYLYX, PCA shall allow inspection of any records regarding the manufacture and the supply of the API (including batch manufacturing records); provided, that, at a minimum, AMYLYX shall be given access generally to the types of documents that the FDA would customarily require for an FDA inspection.

9.3 **Information**. AMYLYX may request at any time, PCA to transmit to AMYLYX the reference number of the DMF as well as the “Applicant Part” of any DMF, describing the process employed by PCA for manufacture of API as envisaged in this Agreement to AMYLYX.

Each Party shall notify the other of any Competent Regulatory Authority's notices of violation or deficiency letters relating to the API and/or the Finished Product, also delivering to the other Party data, information and redacted correspondence received by it from the Competent Regulatory Authority with respect to the API (or Finished Product) and cooperating at its own expense to the reasonable extent asked by the other Party in its response to the Competent Regulatory Authority.

10. **Changes of Specifications, plant of manufacture and/or DMF**

10.1 **By PCA**. In case PCA decides and/or is requested by Competent Regulatory Authority to change the Specifications, process or plant of manufacture, and/or the DMF, there, in either case, it shall first give written notice to AMYLYX, detailing the proposed process changes and obtain prior written approval to make such changes, that cannot be unreasonably denied. If AMYLYX demonstrates within a reasonable period of time after receiving PCA's notice-that the change(s) proposed by PCA may disrupt: (i) the quality, quantity, timeliness or reliability of supply of the API, or (ii) AMYLYX'S ability to use the API in the manner used by AMYLYX prior to any such change(s), or (iii) the regulatory approval in any jurisdiction of Finished Product being marketed and sold by AMYLYX, then AMYLYX shall promptly inform PCA of its determination, and the Parties shall have an obligation to work in good faith to resolve AMYLYX's concerns.

Upon the approval of such changes by AMYLYX, AMYLYX shall exercise commercially reasonable efforts to promptly obtain the relevant approvals of Finished Product from the concerned Competent Regulatory Authority with the modified process of the API, if so required by Law, and shall cooperate in good faith with PCA in order to plan the implementation of the process change.

Until the time AMYLYX obtains the approval as above, or if AMYLYX does not approve the changes proposed by PCA, provided such non approval is based on reasonable grounds, PCA shall continue to manufacture and supply the API as per the last-approved process.

10.2 **By AMYLYX**. In case AMYLYX requires PCA to change the Specifications and/or the DMF or desires that PCA consider changing the process of manufacture, PCA—also based on AMYLYX's detailed report regarding the implementation method of such changes and the purposes of the required changes—shall verify the feasibility of the required changes, based on the Law and on its capacity to adapt the manufacturing process already carried out, and shall notify AMYLYX as soon as possible of the result of such analysis.

PCA will use its commercially reasonable efforts to accommodate such changes, providing AMYLYX with an estimation of the relevant costs and both parties shall cooperate in order to draw up an amendment to the Specifications, if required by such changes. PCA shall carefully consider any recommendation made by AMYLYX with reference to the implementation of the proposed changes. Once PCA and AMYLYX have mutually agreed on an amendment to the Specifications, on the relative timeframes and general principles of proposed changes implementation and the costs therefore, PCA shall implement such changes and AMYLYX shall reimburse PCA for its costs in doing so.

10.3 **By Law**. In the event that Law applicable to PCA' manufacturing activities requires PCA to change the Specifications or process of manufacture, and/or the DMF, PCA shall carry out all the changes pursuant to such Law and AMYLYX, where required, shall assist PCA. Should the changes above involve relevant additional costs or investment in manufacturing the API, the Parties shall negotiate in good faith appropriate compensation of PCA for such additional costs (which may either take the form of a cost reimbursement or a proportional increase of the supply price of the API).

10.4 It remains understood and agreed, that, in any case of change as per articles above, PCA will continue carrying out all of its activities in compliance with mandatory laws applicable to its activities under this Agreement.

11. **Warranty**

11.1 **Warranty by Parties**. Each Party declares and warrants that it has all necessary legal and statutory approvals to conduct its business and to enter into the obligations imposed upon it by this Agreement, including any Exhibits attached hereto.

11.2 **Warranty by PCA**. PCA warrants that the delivered API shall: (i) adhere to description and requisites set forth in this Agreement and in the DMF; (ii) be manufactured strictly in compliance with Law, this Agreement and cGMPs; (iii) meet all Specifications; (iv) be free of security interests and other similar encumbrance; and (v) not to be adulterated or misbranded.

PCA declares that, to the best of its knowledge, neither the API nor the manufacturing process adopted in manufacturing the API infringes any third party valid patent right. PCA expressly disclaims and does not make any warranty or representation that any Finished Product manufactured with the API as a starting material or component or ingredient will not infringe any patents or intellectual property owned by any other party.

This provision *mutatis mutandis* shall apply to any change implemented pursuant to Article 10.

PCA hereby certifies that it is not and has not been debarred and it does not and shall not employ, contract with or retain any person directly or indirectly to perform services that relate to this Agreement or provide any services in any capacity if such person is or has been debarred under 21 U.S.C. 335a (a) or (b) or other equivalent laws, rules, regulations or standards of any other relevant jurisdiction. Upon written request of Amylyx, PCA shall, within [***] business days, provide written confirmation that it has complied with the foregoing obligation. PCA agrees to immediately disclose in writing to Amylyx if it or any employee or agent is debarred, or if any action or investigation is pending or, to the best of PCA's knowledge, is threatened in relation to the debarment of PCA or any person performing services or providing services in any capacity in connection with this Agreement.

Except as expressly set forth in this Agreement, PCA makes no other warranties, express or implied, with respect to the API; thus all other warranties, express or implied, including without limitation, the implied warranties of merchantability and fitness for a particular purpose are hereby disclaimed by PCA.

11.3 **Warranty by AMYLYX**. AMYLYX warrants that it shall pay for the delivered and accepted API, which acceptance shall occur within [***] days of delivery. AMYLYX warrants that, to the extent AMYLYX determines in its sole discretion to develop or commercialize Finished Product in any given jurisdiction of the Territory, AMYLYX shall use its commercially reasonable efforts, at its sole expense, to obtain and maintain any and all approval, registration or like with regard to producing, marketing, selling, labelling or otherwise promoting the Finished Product. Except as otherwise expressly provided by this Agreement, AMYLYX shall be responsible for compliance with Law applicable to manufacturing, marketing, and commercializing the Finished Product.

Except as expressly set forth in this Agreement, AMYLYX makes no other warranties, express or implied, with respect to the Finished Product or otherwise; thus all other warranties, express or implied, including without limitation, the implied warranties of merchantability and fitness for a particular purpose are hereby disclaimed by AMYLYX.

The warranties set forth in Sections 11.1, 11.2 and 11.3 shall be deemed given as of the Effective Date and shall be deemed given as of the date of each delivery of API hereunder, with respect to the API so delivered.

11.4 **Ownership of intellectual property rights**. The Parties acknowledge and agree that the Intellectual Property relating to the API belongs exclusively to PCA and, for the sole and exclusive purpose of the performance of this Agreement in the Territory, including the commercialization of the Finished Product, PCA hereby grants AMYLYX a worldwide and free license to such Intellectual Property relating to the API. Notwithstanding anything to the contrary herein contained, it is agreed and understood that if AMYLYX or any Affiliate or distributor has in its inventory has or under a signed purchase order, any Finished Product following the expiration or termination of this Agreement, then AMYLYX or any Affiliate or distributor shall be allowed to sell and distribute the Finished Product in the normal course provided that all related payments due to PCA are paid in accordance to the terms and conditions of this Agreement.

11.5 The Parties acknowledge and agree that the Intellectual Property relating to the Finished Product exclusively belongs to AMYLYX.

11.6 **Trademarks**. Without prejudice to Section 11.4, each Party undertakes not to use the other Party's name, trade mark or corporate name and not to refer at any time to the business relationships existing between them for advertising, promotional or other purposes without the other Party's prior authorization in writing not to be unreasonably withheld or delayed.

In any case, neither Party shall make any use of the other Party's name, trade mark or corporate name in a way that might result—directly or indirectly—in harm to the other Party's business or products.

12. **Indemnification and Insurance**

12.1 **Indemnification**.

AMYLYX shall indemnify and hold PCA and any of its Affiliates, directors, officers, employees, subcontractors and agents (collectively, the "**PCA Indemnified Parties**") harmless from and against any and all claims, demands, actions, suits, losses, damages, costs, expenses (including reasonable attorney's fees), and liabilities which any PCA Indemnified Party may incur, suffer or be required to pay by reason of: (a) property damage or bodily injury, illness or death of any person caused or alleged to be caused by the use of the API, by AMYLYX or the use, distribution or sale of Finished Product manufactured by or for AMYLYX or (b) AMYLYX' s breach of any of its warranties or obligations under this Agreement, or (c) any patent infringement suit having a favorable outcome for the plaintiff, brought against a PCA Indemnified Party exclusively because of AMYLYX's unlawful use of the API and/or use, marketing, distribution or sale of the Finished Product; provided however that AMYLYX shall have no liability under this paragraph to the extent that such claims, demands, actions, suits, causes of action, damages or expenses occurred as a result of either: (i) PCA's gross negligence, willful misconduct, or breach of its warranties or obligations herein or (ii) claims that the API or the manufacturing process adopted by PCA in manufacturing the API infringes or misappropriates any third party patent or other intellectual property rights.

PCA shall indemnify and hold AMYLYX and any of its Affiliates, directors, officers, employees, subcontractors and agents (collectively the "**AMYLYX Indemnified Parties**") harmless from and against any and all claims, demands, actions, suits, losses, damages, costs, expenses (including reasonable attorney's fees), and liabilities which any AMYLYX Indemnified Party may incur, suffer or be required to pay by reason of (a) property damage or bodily injury, illness or death of any person caused or alleged to

be caused by the API sold by PCA, (b) PCA's breach of any of its warranties or obligations under this Agreement, (c) any patent infringement suit having a favorable outcome for the plaintiff brought against a AMYLYX Indemnified Party exclusively because of the Defective API or (d) any patent or other intellectual property right infringement claim brought against any AMYLYX Indemnified Party relating to AMYLYX's marketing, distribution or sale in the Territory of products incorporating the Defective API, provided, however, that PCA shall have no liability under this paragraph to the extent such claims, demands, actions, suits, causes of action, damages or expenses occurred as a result of either (i) the use made by AMYLYX of the API in combination with other product(s) or excipients in pharmaceutical formulation to the extent such claims, demands, actions, suits, causes of action, damages or expenses arise not out of the unmodified API, but instead as a result of the combination, other product(s) or excipients or formulation, or (ii) modification of the API made by AMYLYX without prior written consent of PCA, or (iii) AMYLYX's gross negligence, willful misconduct, or breach of its warranties or obligations contained herein; or (iv) improper handling or storage of the API by AMYLYX and/or carriage in the event the API is delivered EX WORKS.

EXCEPT TO THE EXTENT THAT A PARTY IS OBLIGATED TO PROVIDE INDEMNIFICATION PURSUANT TO THE FOREGOING PARAGRAPHS WITH RESPECT TO THIRD PARTY CLAIMS AND EXCEPT IN THE CASE OF THE BREACH BY A PARTY OF ITS CONFIDENTIALITY OBLIGATIONS HEREUNDER, NEITHER PARTY SHALL BE LIABLE TO THE OTHER PARTY FOR ANY SPECIAL, INDIRECT, INCIDENTAL OR CONSEQUENTIAL LOSS OR DAMAGE, INCLUDING WITHOUT LIMITATION LOSS OF PROFITS, BUSINESS OR REVENUE, HOWSOEVER CAUSED AND WHETHER SUFFERED BY A PARTY TO THIS AGREEMENT OR ANY OTHER PERSON OR AS A RESULT OF ANY BREACH OF THIS AGREEMENT.

12.2 **Insurance**. Each Party warrants and represents to the other that it currently maintains and covenants that at all times during the term of this Agreement it will maintain a comprehensive general liability insurance policy which: (i) is sufficient to adequately protect against the risks associated with its ongoing business, including the risks which might possibly arise in connection with the transactions contemplated by this Agreement, up to a maximum of [***] USD per occurrence and [***] USD in the aggregate and (ii) provides that it cannot be terminated or cancelled without giving the other- Party [***] days written notice. Each party shall continue to maintain such insurance during the term of this Agreement, and for a period of [***] years following the expiration or termination of this Agreement.

13. **Term and termination**

13.1 **Duration**. This Agreement commence on the Effective Date and shall continue until the fifth (5th) anniversary thereof unless earlier terminated in accordance with the provisions referred below ("**Initial Perm**"). Upon completion of the Initial Term, this Agreement shall automatically renew for an additional period of five (5) years (the "**Renewal Term**", and together with the Initial Term collectively referred to as the "**Term**"), unless earlier terminated in accordance with the terms of this Article 13.

13.2 **Early Termination for Insolvency**. This Agreement (or the relevant Purchase Orders already issued) may be forthwith terminated by either Party immediately upon written notice by one Party to the other, if the other Party is unable to pay its debts, becomes insolvent, files or has filed against it a petition of bankruptcy, makes an assignment for the benefit of creditors or commits any act amounting to a business failure, or if proceedings in bankruptcy or reorganization or for an appointment of a receiver or trustee for or over such Party's property are instituted by or against such Party in any court having jurisdiction thereof, and such proceedings are not vacated, set aside or stayed within [***] days thereof, or if such Party attempts to enter into a general compromise of its liabilities.

13.3 Early termination for non-compliance with Commercial Launch Plan. The Parties agree that if any of the following occurs:

(i) in case of lack of filing by AMYLYX of the regulatory documentation relating to the NDA and the other drug applications under Section 2.2(i), within [***] months following the elapse of the agreed timeline as per the Commercial Launch Plan;

(ii) if the time schedule set out for the development of the Finished Product and/or the commercial production and/or the filing of the relevant dossier in the Territory and/or the commercial launch of the Finished Product in the market is not in line with the Commercial Launch Plan and with the timeline therewith indicated; and/or

(iii) in case the Finished Product be retired for the market for any reasons, other than AMYLYX's breach of its obligations under this Agreement for which paragraph 13.4 shall apply PCA will be entitled to withdraw from the Agreement by giving a [***] months written notice to AMYLYX, being understood that pending such notice the Parties shall discuss in good faith in order to agree on the possible extension of the timeline and/or review of the milestones under the Commercial Launch Plan.

13.4 **Termination by default**. Without prejudice to the above provision under Section 13.3, this Agreement (or the relevant purchase orders already issued) may be terminated by either Party if the other Party fails to perform or otherwise breaches any of its obligations hereunder, by giving written notice of its intent to terminate and stating the grounds therefore. The Party receiving such written notice, shall have [***] days from the receipt thereof to cure the failure or breach. If such failure or breach has not been timely cured, the Party that delivered such notice shall have the right to immediately terminate this Agreement. In no event, however, shall such notice of intention to terminate be deemed to waive any rights to damages or any other remedy which the Party giving notice of breach may have as a consequence of such failure or breach.

13.5 **Effect of termination**. Termination of this Agreement, for whatever reason, shall not affect this provision and the obligations of either Party under Sections 8, 9, 11, 12, 14 and 16, all of which shall remain and continue in full force and effect.

13.6 **Alternative Supplier**. In the event that: (a) PCA fails to fulfil its obligations under this Agreement for whatever reason or (b) this Agreement is terminated by the mutual agreement of the Parties or pursuant to Sections 13.2 or 13.4 owing to a default by PCA, AMYLYX shall have the right to: (i) obtain all API from an alternate supplier, (ii) produce all API itself until such failure is corrected to the reasonable satisfaction of AMYLYX. In the case referred to in point (i) above, upon request, AMYLYX, will receive PCA's assistance and cooperation in good faith in finding and qualifying an alternative source of supply. In the event PCA has reason to believe that it will be unable to fill an order for the API for a period lasting more than [***] months, PCA shall immediately notify AMYLYX and the provisions of this Section 13.6 shall apply. The choice of the alternative supplier shall be at the sole discretion of AMYLYX. AMYLYX has the option to reinstate its orders for the API solely with PCA, except for orders and firm purchase commitments previously placed with the alternate supplier, upon notification and the provision of reasonable evidence from PCA that it has restored its ability to supply the API in accordance with the terms and conditions of this Agreement.

14. Confidentiality

Confidential Information. In carrying out this Agreement, each Party may have access to Confidential Information of the other Party. It is understood and agreed that such access is exclusively intended for the purposes of the performance of this Agreement and does not amount, directly and/or indirectly, to an assignment for any reasons of such Confidential Information to the other Party.

Confidential Information shall not include: (i) information that either is or becomes lawfully publicly available for reasons unrelated to the Parties' activity or inaction; (ii) information that has been lawfully communicated by a third party without any confidentiality obligations; (iii) information that has been developed independently by the other Party as established by that Party's competent written records; (iv) information that has been made public by Law; or (v) information that the Party can prove was already known before he received it.

14.1 **Confidentiality undertakings**. The Parties will strictly safeguard all Confidential Information, take suitable measures and handle that information with a high measure of diligence throughout the duration of this Agreement as well as for [***] years thereafter. If the Confidential Information is identified as a trade secret, such obligation of confidentiality and non-disclosure shall survive the expiration or termination of such [***] year period for so long as such Confidential Information remains a trade secret.

The Parties also undertake not to communicate Confidential Information to others, except where this is required under the Law, any Competent Regulatory Authority, competent judicial authorities or other competent authorities, or serves purposes that are closely related to the performance of this Agreement, and then only upon the giving of advance notice to the other Party, where it is legally possible to do so, to permit the Party to obtain appropriate protections against the further disclosure of its Confidential Information. The Parties additionally undertake to adopt all reasonable precautions to ensure that their own employees and/or collaborators comply with the provisions made herein.

A Party may not disclose Confidential Information to any entity whatsoever apart from its own employees, representatives, and professional consultants requiring this information, on a strictly *"need to know basis"*, provided that they are directly involved in the project. In such a case the Party disclosing Confidential Information shall have the responsibility to ensure that any entity to whom Confidential Information is communicated complies with the conditions laid down herein exactly as if that entity were a party to this Agreement.

Each Party shall return all documents and materials including Confidential Information of the other Party that are in its possession or custody or under its control within [***] days of the request made in writing by the other Party.

15. General provisions

15.1 **Assignment**. Neither Party shall assign this Agreement or any part of it to any third party without the prior written approval of the other Party. Either Party may, however, without any such written approval, assign and transfer this Agreement to a third party in connection with the transfer or sale of all or substantially all of its securities or assets related to the division or the subject business, including for the sake of clarity the sale of or the transfer of title rights into the business of the API or Finished Product, or in the event of its sale, merger or consolidation or change in control. This prohibition on assignment shall in no way restrict or prohibit the assignment or transfer of the rights provided hereunder to any Affiliates.

15.2 **Force Majeure**. For the purpose of this Agreement, Force Majeure means any event that goes beyond the Parties' control and prevents the obligations set out in this Agreement from being fulfilled, in whole or in part, it being unforeseeable, exceptional and utterly unavoidable.

Force Majeure reasons may not be claimed in connection with breaches of contract and delays that took place prior to the occurrence of Force Majeure events.

Should an event of Force Majeure occur, the Parties, in good faith, shall take any reasonable and appropriate steps to minimize the prejudicial effects of any such event on the performance of this Agreement and make any reasonable efforts aimed at avoiding termination of this Agreement.

In the case of a Force Majeure event, the infringing Party shall not be subject to responsibilities for its infringements of obligation set forth in this Agreement which are directly due to the Force Majeure event.

15.3 **No waiver**. Failure by either Party to this Agreement to request execution of the provisions set forth herein will not and may not be regarded as a waiver either of said provisions or of the future compliance with them.

15.4 **Independent Relationship**. Nothing herein contained shall be deemed to create an agency, joint venture or partnership relationship between the Parties. Neither Party shall have any power to enter into any contract or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind the other Party in any respect whatsoever.

15.5 **Notices**. Any notice provided for in this Agreement shall be given to the respective Parties at the following addresses by certified or registered mail, facsimile with confirmation or sent by an internationally recognized overnight courier service.

If to AMYLYX to:

Amylyx Pharmaceuticals, Inc.43 Thorndike Street
Cambridge, MA 02141
Attention: [***]
Email: [***]

With a copy which shall not constitute notice to:

Rubin and Rudman LLP
53 State Street, 15th Floor
Boston, MA 02109
Attention: [***]
Email: [***]

If to PCA, to:
PCA S.p.A.
Via Novi 78
15060 Basaluzzo (AL)
Attention:
Facsimile No.: [***]

Notice shall be deemed given as of [***] days from the date of mailing. In case of need or urgency the notices may be given by fax or telegram and shall be confirmed by the means set forth in this Article.

15.6 **Joint Liability**. This Agreement is made by and for the benefit of each Party and each Party's Affiliates. Each Party and its Affiliates shall together be jointly and severally liable to the other Party for the performance of the obligations set forth herein.

15.7 **No Third Party Rights**. A person who is not a party to this Agreement shall not have any rights under or in connection with it by virtue of the Contracts (Rights of Third Parties) Act 1999, except where such rights are expressly granted hereunder.

16. Governing law and dispute resolution

16.1 **Governing Law**. This Agreement shall be governed, interpreted and construed in accordance with the laws of England and Wales, without regard to the conflict of laws, rules, or principles thereof.

16.2 **Exclusive Jurisdiction**. Any disputes arising out of or in connection with this Agreement or the interpretation, breach or termination thereof, which cannot be resolved by mutual agreement between the Parties within [***] days after the notification of the dispute, shall be submitted to the jurisdiction of the competent courts of London, England.

REMAINDER OF THIS PAGE LEFT INTENTIONALLY BLANK

This Agreement has been negotiated on clause by clause basis by the Parties and it is not a standard form of either Party.

IN WITNESS WHEREOF, PCA and AMYLYX have caused this Agreement to be executed as of the Effective Date by their duly authorized officers.

PCA S.p.A

/s/ Paolo Oligeri
By: Paolo Oligeri
Title Managing Director
Hereunto Duly Authorized

AMYLYX PHARMACEUTICALS, INC.

/s/ Justin Klee
By: Justin Klee
Title: President
Hereunto Duly Authorized

EXHIBIT A

UNIT PRICE

Unit Pricing Table for API ordered and intended for delivery in USD.

[***]	[***]

EXHIBIT B

SPECIFICATIONS OF THE API

PRODOTTI CHIMICI E ALIMENTARI S.p.A.

Via Novi, 78—Basaluzzo—Italy

TAUROURSODEOXYCHOLIC ACID

Product code: [***] *Spec. code:* [***]

Customer: AMYLYX Cambridge, MA (USA)

Test	Requirement	U.M.
Description	[***]	
Solubility	[***]	
Identification	[***]	
Specific optical rotation	[***]	*
Water content (by K.F.)	[***]	%
pH	[***]	
Sulphated ash	[***]	%
Heavy metals	[***]	ppm
Assay	[***]	% o.d.b
[***]	[***]	%
[***]	[***]	%
[***]	[***]	%
Other unid.rel.subs.Tot (TLC)	[***]	%
Residual solvents: [***]	[***]	

Date of issue:
Edition:
Approved by Qualified Person: _____
Approved by QC/RA: _____

EXHIBIT C

COMMERCIAL LAUNCH PLAN

Amylyx's NDA Submission and Commercial Launch milestones:	Anticipated Date:
Pre-NDA Meeting	[***]
NDA submission	[***]
NDA approval	[***]
Commercial Launch	[***]

DEED OF AMENDMENT IN RESPECT OF THE SUPPLY AGREEMENT

THIS DEED is made on the 26th of July 2021 (the ***Deed***)

BETWEEN

AMYLYX PHARMACEUTICALS, INC., a corporation duly organized and existing under the laws of The State of Delaware with a usual place of business at 43 Thorndike St, Cambridge, MA 02141 as represented by [Thomas Holmes] in his capacity as [Global Head, Supply Chain] (***AMYLYX***); and

ICE S.p.A., a joint stock company *(società per azioni*) duly organized and existing under the laws of Italy with registered office at Via Sicilia 8/10, 42122, Reggio Emilia (RE), Italy, as represented by [Enzo Bartoli] in his capacity as [Chairman of the Board of Directors] (***ICE***),

(AMYLYX and ICE, together, the ***Parties*** and each a ***Party***).

WHEREAS

(A) On 9 December 2019, Prodotti Chimici e Alimentari S.p.A. (now merged into ICE) and AMYLYX have entered into the Supply Agreement.

(B) In accordance with the Supply Agreement, the Parties agreed on a certain Commercial Launch Plan attached to the Supply Agreement as Exhibit C thereto (the ***Commercial Launch Plan***).

(C) In light of recent factual developments and discussions between the Parties, the same Parties have come to the conclusion that it would be expedient that the Supply Agreement (with specific reference to the Commercial Launch Plan) is amended as per this Deed.

THE PARTIES AGREE as follows.

1. **INTERPRETATION**

1.1 Words and expressions defined in the Supply Agreement shall have the same meaning when used in this Deed.

2. **AMENDMENTS TO THE SUPPLY AGREEMENT**

2.1 The Commercial Launch Plan (i.e. Exhibit C to the Supply Agreement) shall be amended so to replace the same with the following wording, which shall be deemed as entirely superseding the Commercial Launch Plan (i.e. Exhibit C to the Supply Agreement) originally agreed upon between the Parties and be deemed as it had been part of the Supply Agreement as from the date of execution of this latter:

EXHIBIT C
COMMERCIAL LAUNCH PLAN

First Global Submission Date (NDS, NDA, EMA filing)	***Anticipated Date***
Pre NDA MTG with FDA	[***]
NDS Submission Date	[***]
NDS Anticipated Approval Date	[***]
Commercial Launch	[***]

"

2.2 For the avoidance of doubt, any references made in the Supply Agreement to the Commercial Launch Plan (i.e. Exhibit C to the Supply Agreement) shall be deemed and construed as if they had been made to the Commercial Launch Plan (i.e. Exhibit C to the Supply Agreement) as amended by this Deed.

3. **GOVERNING LAW AND JURISDICTION**

3.1 This Deed and any non-contractual obligations arising out of or in connection with this Deed shall be governed by, and interpreted in accordance with, the laws of England and Wales (without regard to the conflict of laws, rules, or principles thereof.

3.2 Any disputes arising out of or in connection with this Deed or the interpretation, breach or termination thereof, which cannot be resolved by mutual agreement between the Parties within [***] after the notification of the dispute (in accordance with the Supply Agreement), shall be submitted to the jurisdiction of the competent courts of London, England.

DULY DELIVERED as a Deed on the date and year first above written.

* ** ** ** **

Should you agree with this proposal, we would be grateful if you could return to us a copy hereof duly signed and delivered as a deed by your authorised signatory by way of full, irrevocable and unconditional acceptance hereto.

Yours faithfully.

EXECUTED as a **DEED** by

AMYLYX PHARMACEUTICALS, **INC**.

/s/ Joshua Cohen
Joshua Cohen
CEO Amylyx Pharmaceuticals, Inc.
Authorised representative

* * *

For acceptance.

Executed and delivered as a Deed for full, irrevocable and unconditional acceptance.

Yours faithfully,

EXECUTED as a **DEED** by

ICE S.p.A.

/s/ Agostino Barazza
Authorised representative

Exhibit 21.1

SUBSIDIARIES

Subsidiary	Jurisdiction of Incorporation
Amylyx Pharmaceuticals Canada Inc.	Canada
Amylyx Pharmaceuticals EMEA B.V.	Netherlands