UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended June 30, 2023

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-41199

Amylyx Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**46-4600503**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
43 Thorndike St. Cambridge, Massachusetts	**02141**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 682-0917

N/A

(Former name, former address and former fiscal year, if changed since last report)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	AMLX	Nasdaq Global Select Stock Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of August 7, 2023, the registrant had 67,377,743 shares of common stock, $0.0001 par value per share, outstanding.

AMYLYX PHARMACEUTICALS, INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED JUNE 30, 2023

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or Quarterly Report, contains forward-looking statements which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would” or the negative of these terms or other comparable terminology. These statements are not guarantees of future results or performance and involve substantial risks and uncertainties. Forward-looking statements in this Quarterly Report include, but are not limited to, express or implied statements about:

- our ability to maintain existing and obtain additional regulatory approvals of AMX0035 and any future product candidates;
- our ability to successfully commercialize and market AMX0035 and any future product candidates, if approved, and the timing of any commercialization and marketing efforts;
- our ability to contract with third-party suppliers, manufacturers and other service providers and their ability to perform adequately and to produce sufficient quantities of clinical and commercial supplies;
- the potential market size, opportunity, demand and growth potential for AMX0035 and any future product candidates, if approved;
- our ability to build and maintain 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, including our Phase 3 clinical trial of AMX0035 for the treatment of amyotrophic lateral sclerosis, or ALS, known as the PHOENIX trial, our Phase 3 clinical trial of AMX0035 for the treatment of progressive supranuclear palsy, or PSP, known as the ORION trial, and our Phase 2 clinical trial of AMX0035 for the treatment of Wolfram syndrome, known as the HELIOS trial, 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 continue to advance AMX0035 and advance any future product candidates into, and successfully complete, clinical trials;
- our ability to successfully 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, including the timing and outcome of a decision by the European Medicines Agency, or the EMA, regarding whether to approve AMX0035 for the treatment of adults with ALS following a re-examination procedure;
- the pricing and reimbursement of AMX0035 in the U.S., Canada and in any other jurisdictions in which AMX0035 is approved, if any, and of any other product candidates, if approved;
- the rate and degree of market acceptance of AMX0035 and any future product candidates by physicians, patients, third-party payors and others in the medical community;
- 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, including any regulatory developments;
- our estimates regarding expenses, revenue, capital requirements, cash runway and future and needs for additional financing;
- our financial performance;
- the effect of global economic uncertainty and financial market volatility caused by economic effects of rising inflation and interest rates, the COVID-19 pandemic or any future outbreak of any highly infectious or contagious diseases, geopolitical instability, changes in international trade relationships and military conflicts, such as the ongoing conflict between Russia and Ukraine, on any of the foregoing or other aspects of our business or operations; and

- other statements about future events, including those listed under the section entitled “Risk factors”.

Any forward-looking statements in this Quarterly Report reflect our current views with respect to future events and with respect to our future financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those described under the section titled “Risk Factors” and elsewhere in this Quarterly Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

All of our forward-looking statements are as of the date of this Quarterly Report only. In each case, actual results may differ materially from such forward-looking information. We can give no assurance that such expectations or forward-looking statements will prove to be correct. An occurrence of or any material adverse change in one or more of the risk factors or risks and uncertainties referred to in this Quarterly Report or included in our other public disclosures or our other periodic reports or other documents or filings filed with or furnished to the Securities and Exchange Commission, or the SEC, could materially and adversely affect our business, prospects, financial condition and results of operations. Except as required by law, we do not undertake or plan to update or revise any such forward-looking statements to reflect actual results, changes in plans, assumptions, estimates or projections or other circumstances affecting such forward-looking statements occurring after the date of this Quarterly Report, even if such results, changes or circumstances make it clear that any forward-looking information will not be realized. Any public statements or disclosures by us following this Quarterly Report that modify or impact any of the forward-looking statements contained in this Quarterly Report will be deemed to modify or supersede such statements in this Quarterly Report.

We may from time to time provide estimates, projections and other information concerning our industry, the general business environment, and the markets for certain diseases, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events, circumstances or numbers, including actual disease prevalence rates and market size, may differ materially from the information reflected in this Quarterly Report. Unless otherwise expressly stated, we obtained this industry, business information, market data, prevalence information and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources, in some cases applying our own assumptions and analysis that may, in the future, prove not to have been accurate.

TRADEMARKS

Solely for convenience, our trademarks and trade names in this report are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that we will not assert, to the fullest extent under applicable law, our rights thereto.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)
(Unaudited)

	June 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 247,760	$ 62,526
Short-term investments	109,516	284,419
Accounts receivable, net	33,473	15,306
Inventories	24,858	9,769
Prepaid expenses and other current assets	11,787	10,113
Total current assets	427,394	382,133
Property and equipment, net	2,637	2,611
Restricted cash equivalents	719	719
Operating lease right-of-use assets	4,643	5,524
Long-term inventories	17,729	—
Other assets	466	466
Total assets	$ 453,588	$ 391,453
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 24,695	$ 6,257
Accrued expenses	37,241	38,312
Operating lease liabilities, current portion	2,146	2,040
Total current liabilities	64,082	46,609
Operating lease liabilities, net of current portion	3,141	4,237
Total liabilities	67,223	50,846
Commitments and contingencies (Note 13)		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized no shares issued or outstanding	—	—
Common stock, $0.0001 par value; 300,000,000 shares authorized; 67,354,417 and 66,512,011 shares issued and outstanding as of June 30, 2023 and December 31, 2022, respectively	7	7
Additional paid-in capital	717,003	694,906
Accumulated deficit	(330,573)	(354,220)
Accumulated other comprehensive loss	(72)	(86)
Total stockholders' equity	386,365	340,607
Total liabilities, redeemable convertible preferred stock and stockholders' equity	$ 453,588	$ 391,453

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Product revenue, net	$ 98,216	$ —	$ 169,644	$ —
Operating expenses:				
Cost of sales	5,580	—	10,863	—
Research and development	29,044	24,259	53,236	45,723
Selling, general and administrative	43,391	29,994	87,397	56,344
Total operating expenses	78,015	54,253	151,496	102,067
Income (loss) from operations	20,201	(54,253)	18,148	(102,067)
Other income, net:				
Interest income	3,887	402	7,605	533
Other expense, net	(81)	(42)	(343)	(61)
Total other income, net	3,806	360	7,262	472
Income (loss) before income taxes	24,007	(53,893)	25,410	(101,595)
Provision for income taxes	1,933	174	1,763	320
Net income (loss)	$ 22,074	$ (54,067)	$ 23,647	$ (101,915)
Net income (loss) per share attributable to common stockholders				
Basic	$ 0.33	$ (0.93)	$ 0.35	$ (1.85)
Diluted	$ 0.31	$ (0.93)	$ 0.34	$ (1.85)
Weighted-average shares used in computing net income (loss) per share attributable to common stockholders				
Basic	67,233,617	58,275,903	66,976,871	54,958,537
Diluted	70,132,040	58,275,903	70,471,821	54,958,537

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)
(in thousands)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Net income (loss)	$ 22,074	$ (54,067)	$ 23,647	$ (101,915)
Other comprehensive income (loss):				
Foreign currency translation gain (loss)	(18)	58	60	(10)
Net unrealized loss on investments held	(79)	(103)	(46)	(195)
Other comprehensive income (loss)	(97)	(45)	14	(205)
Comprehensive income (loss)	$ 21,977	$ (54,112)	$ 23,661	$ (102,120)

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY
(in thousands, except share data)
(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	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Capital			
Balance as of December 31, 2022	—	$ —	—	$ —	—	$ —	—	$ —	66,512,011	$ 7	$ 694,906	$ (86)	$ (354,220)	$ 340,607
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	451,298	—	2,777	—	—	2,777
Issuance of common stock upon vesting of RSUs	—	—	—	—	—	—	—	—	132,294	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	7,580	—	—	7,580
Other comprehensive income	—	—	—	—	—	—	—	—	—	—	—	111	—	111
Net income	—	—	—	—	—	—	—	—	—	—	—	—	1,573	1,573
Balance as of March 31, 2023	—	$ —	—	$ —	—	$ —	—	$ —	67,095,603	$ 7	$ 705,263	$ 25	$ (352,647)	$ 352,648
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	226,138	—	1,687	—	—	1,687
Issuance of common stock upon vesting of RSUs	—	—	—	—	—	—	—	—	32,676	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	10,053	—	—	10,053
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(97)	—	(97)
Net income	—	—	—	—	—	—	—	—	—	—	—	—	22,074	22,074
Balance as of June 30, 2023	—	$ —	—	$ —	—	$ —	—	$ —	67,354,417	$ 7	$ 717,003	$ (72)	$ (330,573)	$ 386,365

	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	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' (Deficit) Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Capital			
Balance as of December 31, 2021	6,289,609	$ 7,675	14,496,835	$ 64,387	13,150,430	$ 134,791	3,170,585	$ 32,498	7,020,487	$ 1	$ 4,667	$ 9	$ (155,845)	$ (151,168)
Conversion of preferred stock into common stock upon initial public offering	(6,289,609)	(7,675)	(14,496,835)	(64,387)	(13,150,430)	(134,791)	(3,170,585)	(32,498)	39,474,330	4	239,347	—	—	239,351
Issuance of common stock upon initial public offering, net of issuance costs of $19,639	—	—	—	—	—	—	—	—	11,369,369	1	196,378	—	—	196,379
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	4,392	—	—	4,392
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(160)	—	(160)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(47,848)	(47,848)
Balance as of March 31, 2022	—	$ —	—	$ —	—	$ —	—	$ —	57,864,186	$ 6	$ 444,784	$ (151)	$ (203,693)	$ 240,946
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	669,040	—	248	—	—	248
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	5,707	—	—	5,707
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(45)	—	(45)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(54,067)	(54,067)
Balance as of June 30, 2022	—	$ —	—	$ —	—	$ —	—	$ —	58,533,226	$ 6	$ 450,739	$ (196)	$ (257,760)	$ 192,789

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)
(Unaudited)

	Six Months Ended June 30,	
	2023	**2022**
Cash flows provided by (used in) operating activities:		
Net income (loss)	$ 23,647	$ (101,915)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Stock-based compensation expense	17,451	10,099
Depreciation expense	485	140
Accretion of investment discounts, net	(4,886)	(107)
Changes in operating assets and liabilities:		
Accounts receivable, net	(18,167)	—
Inventories	(32,636)	—
Interest receivable	(356)	(13)
Prepaid expenses and other current assets	(1,318)	(4,053)
Operating lease right-of-use assets	881	790
Other assets	—	(456)
Accounts payable	18,455	3,755
Accrued expenses	971	4,784
Operating lease liabilities	(990)	(120)
Net cash provided by (used in) operating activities	3,537	(87,096)
Cash flows provided by (used in) investing activities:		
Purchases of property and equipment	(528)	(1,447)
Purchases of investments	(9,756)	(154,313)
Proceeds from maturities of short-term investments	189,500	60,911
Net cash provided by (used in) investing activities	179,216	(94,849)
Cash flows provided by financing activities:		
Proceeds from initial public offering	—	200,897
Initial public offering costs paid	—	(1,518)
Follow-on offering costs paid	(136)	—
Proceeds from exercise of stock options	5,663	248
Withholding taxes paid on stock-based awards	(3,103)	—
Net cash provided by financing activities	2,424	199,627
Effect of exchange rate changes on cash, cash equivalents and restricted cash equivalents	57	(203)
Net increase in cash, cash equivalents and restricted cash equivalents	185,234	17,479
Cash, cash equivalents and restricted cash equivalents, beginning of period	63,245	50,380
Cash, cash equivalents and restricted cash equivalents, end of period	$ 248,479	$ 67,859
Supplemental disclosure of cash flow information:		
Unrealized loss on short-term investments	$ 46	$ 195
Taxes withheld on stock-based awards included in accrued expenses	$ 67	$ —
Purchases of property and equipment included in accounts payable	$ 81	$ 76
Right-of-use assets and liabilities upon ASC 842 adoption	$ —	$ 2,201
Right-of-use assets obtained in exchange for lease liabilities	$ —	$ 4,958
Movement of deferred offering costs to equity	$ —	$ 4,518
Initial public offering costs included in accounts payable and accrued expenses	$ —	$ 526
Conversion of preferred stock to common stock upon initial public offering	$ —	$ 239,351
Income taxes paid	$ 2,562	$ —

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

1. NATURE OF THE BUSINESS

Amylyx Pharmaceuticals, Inc., together with its wholly owned subsidiaries, known as Amylyx or the Company, is a commercial-stage biotechnology company with a mission to one day end the suffering caused by neurodegenerative diseases. The Company is pursuing amyotrophic lateral sclerosis, or ALS, as its first indication and is focused on the development and potential commercialization of AMX0035 for ALS globally. Amylyx' first product, RELYVRIO® (sodium phenylbutyrate and taurursodiol, also known as ursodoxicoltaurine), also known as AMX0035, is approved by the U.S. Food and Drug Administration, or the FDA, for the treatment of ALS in adults in the U.S. AMX0035 is also approved with conditions by Health Canada and marketed as ALBRIOZA™ for the treatment of ALS in Canada. In June 2023, the Company announced that the Committee for Medicinal Products for Human Use, or CHMP, of the European Medicines Agency, or EMA, adopted a negative opinion on the application for conditional marketing authorisation of AMX0035, under the trade name ALBRIOZA®, for the treatment of adults with ALS in the European Union, or EU. Following the Company's request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation. The re-examination procedure is an approximately four-month process, which includes the appointment of a different rapporteur and co-rapporteur from the initial evaluation. At the end of the re-examination, the EMA will review its initial opinion before issuing a final recommendation, which is expected in the fall of 2023. The Company is also developing AMX0035 for other neurodegenerative diseases by leveraging its scientific expertise and relationships in the neurodegenerative space. The Company dosed the first participant in the HELIOS trial, a Phase 2 trial of AMX0035 for the treatment of Wolfram syndrome, in April 2023 and plans to initiate the ORION trial, a global, pivotal Phase 3 trial of AMX0035 for the treatment of progressive supranuclear palsy, or PSP by the end of 2023. The Company is also advancing additional drug candidates for neurodegenerative diseases including AMX0114, an antisense oligonucleotide targeting Calpain-2, a key protein in axonal degeneration, among others.

Risks and Uncertainties

The Company is subject to risks and uncertainties common to companies in the biotechnology industry, including, but not limited to, the outcome of preclinical studies and clinical trials, market acceptance and the successful commercialization of its approved products ALBRIOZA, which received marketing authorization with conditions in Canada in June 2022, and RELYVRIO, which was approved by the FDA in the U.S. in September 2022, potential difficulties with or delays in timing with respect to the regulatory approval processes of the EMA and other comparable foreign authorities, 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 economic challenges caused by the COVID-19 pandemic or other public health concerns and economic uncertainty in various global markets caused by geopolitical instability and conflict. The Company and its contractors may experience disruptions in supply of items that are essential for its research and development and commercial 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 additional or future product candidates.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Significant Accounting Policies

The Company's significant accounting policies are disclosed in the audited consolidated financial statements for the year ended December 31, 2022 and the notes thereto, which are included in the Company's most recent Annual Report on Form 10-K. Since the date of those consolidated financial statements, there have been no material changes to its significant accounting policies, with the exception of significant accounting policies related to the adoption of the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 326, *Financial Instruments—Credit Losses*, or ASC 326, effective January 1, 2023, as described below.

Basis of Presentation and Consolidation

The accompanying condensed consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the U.S., or GAAP, and include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. In the opinion of the Company's management, all normal and recurring adjustments necessary for a fair presentation have been reflected. Any reference in these notes

to applicable guidance is meant to refer to the authoritative GAAP as found in the ASC, and Accounting Standards Update, or ASU, of the FASB.

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 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: gross-to-net, or GTN, adjustments; recoverability of inventories, including those produced in preparation for product launches; accrued expenses; stock option valuations; valuation allowance for deferred tax assets and research and development expenses.

Recently Adopted Accounting Pronouncements

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326) Measurement of Credit Losses on Financial Instruments*, or ASU 2016-13. The provisions of ASU 2016-13 modify the impairment model to utilize an expected loss methodology in place of the currently used incurred loss methodology and require a consideration of a broader range of reasonable and supportable information to inform credit loss estimates. 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. The Company adopted ASU 2016-13 effective January 1, 2023, with no material impact on its consolidated financial statements and related disclosures.

3. PRODUCT REVENUE, NET

To date, the Company's only source of product revenue has been from the sales of RELYVRIO, known as ALBRIOZA in Canada. Significant judgment is required in estimating GTN adjustments considering historical experience, payer channel mix, current contract prices, unbilled claims, processing time lags, inventory levels in the distribution channel and estimated product returns. The following table reconciles gross product revenue to net product revenue (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Product revenue, gross	$ 108,405	$ —	$ 192,958	$ —
GTN adjustments	(10,189)	—	(23,314)	—
Product revenue, net	$ 98,216	$ —	$ 169,644	$ —

The activity and ending reserve balance for GTN adjustments were as follows for the six months ended June 30, 2023 (in thousands):

	Chargebacks and Cash Discounts	Medicaid and Medicare Rebates	Other Rebates, Returns, Discounts and Adjustments	Total
Ending balance at December 31, 2022	$ 648	$ 1,992	$ 1,664	$ 4,304
Provision related to sales in the current year	10,053	6,516	7,147	23,716
Adjustments related to prior period sales	—	(236)	(166)	(402)
Credits and payments made	(5,831)	(4,294)	(5,988)	(16,113)
Ending balance at June 30, 2023	$ 4,870	$ 3,978	$ 2,657	$ 11,505

Included in the ending reserve balance for GTN adjustments are chargebacks resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to customers who directly purchase the product from the Company, discounts to customers for prompt payment and estimates for product returns. Chargebacks, discounts and returns are recorded as reductions of accounts receivable, net on the condensed consolidated balance sheets. In addition, included in the ending reserve balance for GTN adjustments are Medicaid and Medicare rebates, other rebates for obligations under voluntary patient

assistance programs, and accrued fees payable to customers. Medicaid and Medicare rebates, other rebates and fees are recorded as a component of accrued expenses on the condensed consolidated balance sheets.

4. SHORT-TERM INVESTMENTS

The Company determines the appropriate classification of marketable securities at the time of purchase and reevaluates such designation at each balance sheet date. The Company has classified all of its marketable securities at June 30, 2023 as “available-for-sale” pursuant to ASC 320, *Investments – Debt and Equity Securities*. The Company records available-for-sale securities at fair value, with the unrealized gains and losses included as a separate component of other accumulated comprehensive income (loss). There were no realized gains or losses recognized during the three and six months ended June 30, 2023 and 2022.

The Company adjusts the cost of available-for-sale debt securities for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion are included in interest income. The cost of securities sold is based on the specific identification method. The Company includes interest and dividends on securities classified as available-for-sale in interest. Accrued interest receivable relating to the Company's available-for-sale securities is presented within prepaid expenses and other current assets in the accompanying condensed consolidated balance sheets, and amounted to $0.8 million and $0.5 million at June 30, 2023 and December 31, 2022, respectively.

The following is a summary of available-for-sale securities with unrealized losses for less than 12 months as of June 30, 2023 and December 31, 2022 (in thousands):

	June 30, 2023		December 31, 2022	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Treasury notes	$ 27,762	$ (24)	$ 27,159	$ (14)
Treasury bills	—	—	9,839	(2)
Commercial paper	46,371	(26)	—	—
Corporate debt securities	15,516	(11)	33,486	(55)
Agency bonds	9,870	(20)	—	—
Total available-for-sale securities in an unrealized loss position	$ 99,519	$ (81)	$ 70,484	$ (71)

At June 30, 2023, the Company's security portfolio consisted of 15 securities related to investments in debt securities available-for-sale, of which 14 securities were in an unrealized loss position. There were no securities in an unrealized loss position for greater than 12 months as of June 30, 2023. The contractual terms of these investments do not permit the issuer to settle the securities at a price less than the amortized cost bases of the investments. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost bases. The Company did not record an allowance for credit losses as of June 30, 2023.

Prior to January 1, 2023, the Company evaluated 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 were also included in other income, net. When assessing short-term investments for other-than-temporary declines in value, the Company considered 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. The Company determined it did not hold any investments with any other-than-temporary impairment as of December 31, 2022.

Short-term investments, which are classified as available-for-sale, consisted of the following (in thousands):

Balance at June 30, 2023:	Amortized Cost Basis	Unrealized Gain	Unrealized Loss	Fair Value
Treasury notes	$ 27,786	$ —	$ (24)	$ 27,762
Commercial paper	46,397	—	(26)	46,371
Corporate debt securities	25,521	3	(11)	25,513
Agency bonds	9,890	—	(20)	9,870
Total short-term investments	$ 109,594	$ 3	$ (81)	$ 109,516

Balance at December 31, 2022:	Amortized Cost Basis	Unrealized Gain	Unrealized Loss	Fair Value
Treasury notes	$ 27,173	$ —	$ (14)	$ 27,159
Treasury bills	59,326	10	(2)	59,334
Commercial paper	134,375	—	—	134,375
Corporate debt securities	58,795	13	(55)	58,753
Agency bonds	4,781	17	—	4,798
Total short-term investments	$ 284,450	$ 40	$ (71)	$ 284,419

5. INVENTORIES

Inventories consisted of the following (in thousands):

	June 30, 2023	December 31, 2022
Raw materials	$ 28,915	$ 7,151
Work in process	8,702	1,681
Finished goods	4,970	937
Total inventories	$ 42,587	$ 9,769

The Company capitalizes inventory costs associated with its products after regulatory approval when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized. As of June 30, 2023, the Company had $12.0 million of inventory on hand that was acquired prior to regulatory approvals. This inventory was expensed to research and development as the future economic benefit was not probable. The Company began to capitalize inventory costs upon receipt of regulatory approvals in 2022. Finished goods have a shelf life of 12-18 months from the date of manufacture.

6. ACCRUED EXPENSES

Accrued expenses consisted of the following (in thousands):

	June 30, 2023	December 31, 2022
Accrued external research and development	$ 9,298	$ 8,424
Accrued benefits and incentive compensation	7,946	15,231
Accrued manufacturing	4,339	4,596
Accrued consulting and other professional fees	5,407	4,116
Accrued rebates and co-pay assistance	6,456	3,582
Accrued royalties	3,111	1,358
Other accrued expenses	684	1,005
Total accrued expenses	$ 37,241	$ 38,312

7. FAIR VALUE MEASUREMENTS

The following tables present 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 (in thousands):

	June 30, 2023			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 150,723	$ —	$ —	$ 150,723
Short-term investments:				
Treasury notes	27,762	—	—	27,762
Commercial paper	—	46,371	—	46,371
Corporate debt securities	—	25,513	—	25,513
Agency bonds	—	9,870	—	9,870
Total short-term investments	27,762	81,754	—	109,516
Restricted cash equivalents	719	—	—	719
Total financial assets	$ 179,204	$ 81,754	$ —	$ 260,958

	December 31, 2022			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 23,567	$ 9,989	$ —	$ 33,556
Short-term investments:				
Treasury notes	27,159	—	—	27,159
Treasury bills	59,334	—	—	59,334
Commercial paper	—	134,375	—	134,375
Corporate debt securities	—	58,753	—	58,753
Agency bonds	—	4,798	—	4,798
Total short-term investments	86,493	197,926	—	284,419
Restricted cash equivalents	719	—	—	719
Total financial assets	$ 110,779	$ 207,915	$ —	$ 318,694

The Company classifies its money market funds, treasury notes and treasury bills as Level 1 assets under the fair value hierarchy, as these assets have been valued using quoted market prices for identical assets in active markets without any valuation adjustment. The Company classifies its commercial paper, corporate debt securities, and agency bonds as Level 2 assets under the fair value hierarchy, as these assets have been valued using information obtained through a third-party pricing service at each balance sheet date, using observable market inputs that may include trade information, broker or dealer quotes, bids, offers, or a combination of these data sources.

8. LEASES

The Company adopted ASC 842 on January 1, 2022. ASC 842 allows the Company to elect a package of practical expedients, which include: (i) an entity need not reassess whether any expired or existing contracts are or contain leases; (ii) an entity need not reassess the lease classification for any expired or existing leases; and (iii) an entity need not reassess any initial direct costs for any existing leases. Another practical expedient allows the Company to use hindsight in determining the lease term when considering lessee options to extend or terminate the lease and to purchase the underlying asset. The Company elected to utilize this package of practical expedients and did not elect the hindsight methodology in its implementation of ASC 842. The Company leases its office facilities under non-cancelable operating leases that expire at various dates through October 2026.

Components of lease expense required by ASC 842 are presented below for the three and six months ended June 30, 2023 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Lease cost				
Operating lease cost	$ 544	$ 544	$ 1,088	$ 1,048
Total lease cost	$ 544	$ 544	$ 1,088	$ 1,048

During the six months ended June 30, 2023, the Company made cash payments of $1.2 million for operating leases. Future minimum lease payments under non-cancelable leases as of June 30, 2023, were as detailed below (in thousands):

	June 30, 2023
2023 (remaining 6 months)	$ 1,220
2024	2,478
2025	1,586
2026	476
2027	—
Total undiscounted lease payments	5,760
Less: imputed interest	(473)
Total operating lease liabilities	$ 5,287

As of June 30, 2023, the weighted average remaining lease term was 2.4 years and the weighted average incremental borrowing rate used to determine the operating lease right-of-use assets was 7.3%.

9. INCOME TAXES

For the three months ended June 30, 2023 and 2022, the Company recorded an income tax provision of $1.9 million and $0.2 million, respectively. The income tax provision for each of the three months ended June 30, 2023 and 2022 was primarily driven by the estimated effective tax rate for the year, as well as discrete income tax benefit of $0.1 million and zero, respectively. For the six months ended June 30, 2023 and 2022, the Company recorded an income tax provision of $1.8 million and $0.3 million, respectively. The income tax provision for the six months ended June 30, 2023 and 2022 was primarily driven by the estimated effective tax rate for the year, as well as discrete income tax benefit of $0.4 million and zero, respectively. The income tax provision for the three and six months ended June 30, 2023 includes the release of a portion of the Company's valuation allowance that has been reversed through the annual effective tax rate with respect to amounts expected to be realized through current year taxable income.

10. STOCK OPTION AND GRANT PLAN

Stock Incentive Plan

General Option Information

A summary of option activity for the six months ended June 30, 2023, is as follows:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2022	8,480,950	$ 13.19	8.2	$ 201,765
Granted	2,399,056	$ 31.96		
Exercised	(677,436)	$ 6.59		$ 16,202
Cancelled or forfeited	(210,721)	$ 17.54		
Outstanding at June 30, 2023	9,991,849	$ 18.05	8.2	$ 66,550
Options exercisable as of June 30, 2023	3,172,459	$ 11.01	7.2	$ 34,430
Options unvested as of June 30, 2023	6,819,390	$ 21.32	8.7	$ 32,120
Weighted average grant-date fair value of options granted during the period	$ 21.15			

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 six months ended June 30, 2023 and 2022 was $16.2 million and $6.0 million, respectively.

The total fair value of stock options vested during the six months ended June 30, 2023 and 2022 was $19.9 million and $3.2 million, respectively.

Restricted Stock Unit Activity

A summary of restricted stock unit activity for the six months ended June 30, 2023, is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value
Nonvested as of December 31, 2022	740,297	$ 20.02
Granted	512,878	$ 32.11
Vested	(164,970)	$ 18.42
Forfeited	(26,141)	$ 25.66
Nonvested as of June 30, 2023	1,062,064	$ 25.97

Summary of Stock-Based Compensation Expense

Stock-based compensation expense recorded in the condensed consolidated statements of operations for the three and six months ended June 30, 2023 and 2022, is as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Research and development	$ 2,535	$ 1,460	$ 4,426	$ 2,585
Selling, general and administrative	7,415	4,247	13,025	7,514
Total stock-based compensation expense	$ 9,950	$ 5,707	$ 17,451	$ 10,099

The Company capitalized stock-based compensation expense of $0.1 million and $0.2 million for the three and six months ended June 30, 2023, respectively. The Company did not capitalize any stock-based compensation expense during the three and six months ended June 30, 2022. Stock-based compensation recognized through cost of sales was less than $0.1 million for the three and six months ended June 30, 2023.

The following table summarizes stock-based compensation by type of award (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Stock options	$ 8,115	$ 4,955	$ 14,371	$ 8,863
Restricted stock units	1,835	752	3,080	1,236
Total stock-based compensation expense	$ 9,950	$ 5,707	$ 17,451	$ 10,099

The following table summarizes unrecognized stock-based compensation expense as of June 30, 2023, by type of awards (in thousands), and the weighted-average period over which that expense is expected to be recognized (in years). The total unrecognized stock-based compensation expense will be adjusted for actual forfeitures as they occur.

	June 30, 2023	
	Unrecognized Expense	Weighted-average Recognition Period
Stock options	$ 94,199	3.01
Restricted stock units	$ 24,635	3.31

11. NET INCOME (LOSS) PER SHARE

Net Income (Loss) per Share Attributable to Common Stockholders

Basic earnings per share is computed by dividing net income (loss) by the weighted average number of common shares outstanding during the period. Diluted earnings per share is calculated based on the combined weighted average number of common

shares and potentially dilutive shares, which include the assumed exercise of employee stock options and unvested restricted stock units. In computing diluted earnings per share, the Company utilizes the treasury stock method.

A summary of the numerator and denominators used in the computation of earnings per share follows (in thousands, except share and per share data):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Numerator:				
Net income (loss)	$ 22,074	$ (54,067)	$ 23,647	$ (101,915)
Denominator:				
Weighted-average shares used to compute basic net income (loss) per share	67,233,617	58,275,903	66,976,871	54,958,537
Dilutive effect of employee stock options and restricted stock units	2,898,423	—	3,494,950	—
Weighted-average shares used to compute diluted net income (loss) per share	70,132,040	58,275,903	70,471,821	54,958,537
Net income (loss) per share attributable to common stockholders				
Basic	$ 0.33	$ (0.93)	$ 0.35	$ (1.85)
Diluted	$ 0.31	$ (0.93)	$ 0.34	$ (1.85)

Because the Company reported a net loss attributable to common stockholders for the three and six months ended June 30, 2022, basic and diluted net loss per share attributable to common stockholders were the same. All stock options and restricted stock units were excluded from the computation of diluted weighted-average shares outstanding because such securities would have an antidilutive impact for the three and six months ended June 30, 2022. The following stock options and restricted stock units outstanding at each period end have been excluded from the calculation of diluted net income (loss) per share because their inclusion would have been antidilutive:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Options to purchase common stock	5,831,498	8,528,039	3,198,508	8,528,039
Restricted stock units	567,671	670,013	85,658	670,013
Total excluded common stock equivalents	6,399,169	9,198,052	3,284,166	9,198,052

12. RELATED PARTY TRANSACTIONS

Supplier Agreements

In the ordinary course of business, the Company may purchase materials or supplies or services from entities that are associated with a party that meets the criteria of a related party of the Company. These transactions are reviewed quarterly and to date have not been material to the Company's condensed consolidated financial statements.

13. COMMITMENTS AND CONTINGENCIES

Lease Commitments

The Company has two operating lease agreements for its office space. See Note 8 for additional information.

Letter of Credit

Restricted cash equivalents consist of $0.2 million of cash serving as collateral for a letter of credit issued for the Company's office space, and $0.5 million as collateral for a corporate credit card program. As of June 30, 2023 and December 31, 2022, the Company's restricted cash equivalents balance was $0.7 million on its condensed consolidated balance sheets.

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 agreements with the ALS Association, ALS Finding a Cure Foundation, Alzheimer's Drug Discovery Foundation, Alzheimer's Association and Cure Alzheimer's Fund, or Grantors. Under the terms of the agreements, the Company was granted, in aggregate, $4.3 million. These grants were provided to the Company for the purpose of furthering the research and development of AMX0035 as a therapeutic benefit for ALS and Alzheimer's disease. Under the terms of the arrangements, the Company would receive a 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. During the three and six months ended June 30, 2023, the Company recorded $0.6 million and $3.1 million in royalty expense, respectively, which is included in cost of sales in the condensed consolidated financial statements. As of June 30, 2023, no further royalties remain to be accrued under the grant agreements with the ALS Association and ALS Finding a Cure Foundation.

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 generating transactions associated with the projects for which the grants were used for under the grant agreements. As the conditions that would trigger royalty payments under the agreements have not occurred, no amounts have been recorded in the condensed consolidated financial statements for the three and six months ended June 30, 2023 and 2022.

Purchase Commitments

The Company enters into agreements in the normal course of business with contract manufacturing organizations, or CMOs, for raw material purchases and manufacturing services. As of June 30, 2023, the Company had committed approximately $86.8 million under these agreements related to raw material purchases and manufacturing services, which are expected to be paid through 2025.

14. SUBSEQUENT EVENTS

The Company has evaluated all subsequent events after June 30, 2023 and through the date of this filing, and there were no material subsequent events requiring disclosure, except as described below.

2023 Inducement Plan

In July 2023, the Company's board of directors adopted the Amylyx Pharmaceuticals, Inc. 2023 Inducement Plan, or the Inducement Plan, to grant equity awards to induce highly-qualified prospective officers and employees who are not currently employed by the Company to accept employment and provide them with a proprietary interest in the Company, pursuant to Rule 5635(c)(4) of the Marketplace Rules of the NASDAQ Stock Market, Inc. The Company has reserved 750,000 shares of its common stock that may be issued under the Inducement Plan.

Supplier Agreements

In August 2023, the Company entered into an agreement with a CMO for the purchase of raw materials. Under the terms of the agreement, the Company committed to minimum annual raw material purchases. As a result, the Company's total purchase commitments related to raw material purchases and manufacturing services have increased to approximately $223.3 million, which are expected to be paid through 2028.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following information should be read in conjunction with the condensed consolidated financial information and the notes thereto appearing elsewhere in this Quarterly Report.

This discussion and other parts of this Quarterly Report 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 our most recent Annual Report on Form 10-K, or 2022 Annual Report, 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 one day end the suffering caused by neurodegenerative diseases. We are committed to supporting and creating more moments for the neurodegenerative disease community through the discovery and development of innovative new treatments. Our first U.S. product, RELYVRIO® (sodium phenylbutyrate and taurursodiol), also known as AMX0035, is approved for the treatment of amyotrophic lateral sclerosis, or ALS, in adults. AMX0035 is also approved with conditions by Health Canada and marketed as ALBRIOZA™ for the treatment of ALS in Canada.

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 believe AMX0035 is the first drug candidate to show both a functional and survival benefit in a large-scale clinical trial of participants living with ALS. The results of our Phase 2 clinical trial of AMX0035, known as the CENTAUR trial, were published in the *New England Journal of Medicine*, in two publications in *Muscle & Nerve*, and in the *Journal of Neurology*, *Neurosurgery*, and *Psychiatry.* 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 received marketing authorization with conditions by Health Canada for ALBRIOZA for the treatment of ALS. We announced commercial availability of the product in July 2022. We have submitted to and received from the national reimbursement authorities, 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 INESSS, recommendations regarding reimbursement for ALBRIOZA by the Canadian provincial governments. In June 2023, we announced that we had completed the negotiation process and entered into a Letter of Intent with the pan-Canadian Pharmaceutical Alliance for the terms and conditions under which ALBRIOZA would qualify for reimbursement through federal, provincial, and territorial public drug plans for the treatment of ALS. We are now engaging with federal, provincial, and territorial public drug plans to work on finalizing and signing product listing agreements for public funding of ALBRIOZA by the end of 2023.

We received approval by the U.S. Food and Drug Administration, or the FDA, for RELYVRIO in September 2022, and commercial product was first available in October 2022.

We are also actively pursuing regulatory approval of AMX0035, under the trade name ALBRIOZA®, for the treatment of ALS in the European Union, or EU. In June 2023, we announced that the Committee for Medicinal Products for Human Use, or CHMP, of the European Medicines Agency, or EMA, adopted a negative opinion on the application for conditional marketing authorisation of AMX0035 for the treatment of adults with ALS in the EU. Following our request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation. The re-examination procedure is an approximately four-month process, which includes the appointment of a different rapporteur and co-rapporteur from the initial evaluation. At the end of the re-examination, the EMA will review its initial opinion before issuing a final recommendation, which is expected in the fall of 2023.

AMX0035 is a dual UPR-Bax apoptosis inhibitor composed of PB and TURSO (also known as TUDCA). Through the resolution of the UPR and by inhibiting translocation of the 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, or ER, 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, or MS. 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.

In November 2021, we initiated a Phase 3 clinical trial of AMX0035 for the treatment of ALS, known as the PHOENIX trial, at clinical trial sites in the U.S. and Europe. On February 2, 2023, we announced completion of enrollment in PHOENIX, which

enrolled 664 participants. We anticipate topline results from the PHOENIX trial in mid-2024. This trial is designed to provide further data evaluating the safety and efficacy of AMX0035 over 48 weeks for the treatment of ALS to further support our global regulatory efforts. Participants completing the 48-week trial have the option to enroll in an open label extension, or OLE, phase. During this phase, all participants receive AMX0035, and continued safety and efficacy measures will be assessed.

In April 2023, we announced that the first participant was dosed in the HELIOS trial, a Phase 2 exploratory open-label proof of biology study assessing the effect of AMX0035 in adults with Wolfram syndrome, with co-primary endpoints examining measures of safety and tolerability, and the effect of AMX0035 during a 4 hour mixed-meal tolerance test. Secondary outcome measures include various measures of endocrinological, neurological and ophthalmologic function. We anticipate topline results from the HELIOS trial in 2024.

We plan to initiate the ORION trial, a global, pivotal Phase 3 trial of AMX0035 for the treatment of progressive supranuclear palsy, or PSP by the end of 2023. There are currently no approved therapies for the treatment of PSP and the disease is reported to affect seven in 100,000 people worldwide. PSP is a rare neurological disorder that affects body movements, walking and balance, and eye movement and is characterized by widespread neurodegeneration associated with tau protein deposition in subcortical regions of the brain. Based on preclinical data and biomarker analyses from the Phase 2 PEGASUS trial of AMX0035 in AD, AMX0035 was shown to significantly lower levels of tau and other markers of neurodegeneration. We intend to enroll approximately 600 adult participants across the U.S., Canada, Europe, and Japan.

In addition, we continue to study other compounds targeting ALS and other neurodegenerative diseases. Outside of AMX0035, the IND-enabling studies of AMX0114, an internally developed antisense oligonucleotide to target Calpain-2, are ongoing. Calpain-2 is a critical effector of axonal degeneration, and there is substantial preclinical data implicating this target in ALS and neuronal degeneration.

Since inception, we have devoted substantially all of our efforts to research and development, pre-commercialization and commercialization activities, including recruiting management and technical staff, raising capital, producing materials for preclinical studies and clinical trials, and building infrastructure to support such activities. As of June 30, 2023, we have funded our operations primarily through public offerings of our common stock, private sales of preferred stock, convertible notes, and more recently through revenue from sales of RELYVRIO and ALBRIOZA in the U.S. and Canada, respectively. We have also generated grant 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 Grantors.

Prior to 2023, we had incurred operating losses and as of June 30, 2023, we had an accumulated deficit of $330.6 million. These losses resulted primarily from costs incurred in connection with research and development activities and general and administrative costs associated with our operations. We expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution of our approved products. We may incur significant losses and our financial results will be highly dependent upon our successful commercialization of RELYVRIO in the U.S. We will continue to incur significant expenses as we advance AMX0035 and any future product candidates through preclinical and clinical development, setup and initiate additional trials, 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.

We also expect to continue 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 may need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate sufficient revenue from product sales to sustain profitability, 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 our current operating plan will be achieved or that additional funding, if required, will be available on terms acceptable to us, or at all.

As of June 30, 2023, we had cash, cash equivalents and short-term investments of $357.3 million. We believe that the revenue we generate from commercial sales of AMX0035 in the U.S. and Canada and our existing cash, cash equivalents and short-term investments as of June 30, 2023, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least one year from the date of this Quarterly Report. 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 Macroeconomic Factors

The development of AMX0035 and any future product candidates could be disrupted and materially adversely affected in the future by any pandemic or calamity. In addition, economic uncertainty in various global markets, including the U.S. and Europe, caused by political instability and conflict, such as the ongoing conflict in Ukraine, and economic challenges caused by global pandemics or other public health events, have led to market disruptions, including significant volatility in commodity prices, credit and capital market instability and supply chain interruptions, which have caused record inflation globally. Our business, financial condition and results of operations could be materially and adversely affected by further negative impact on the global economy and capital markets resulting from these global economic conditions, particularly if such conditions are prolonged or worsen.

Although, to date, our business has not been materially impacted by these global economic and geopolitical conditions, it is impossible to predict the extent to which our operations will be impacted in the short and long term, or the ways in which such instability could impact our business and results of operations. The extent and duration of these market disruptions, whether as a result of the military conflict between Russia and Ukraine and effects of the Russian sanctions, geopolitical tensions, record inflation or otherwise, are impossible to predict, but could be substantial. Any such disruptions may also magnify the impact of other risks described in this report.

Components of Our Results of Operations

Product Revenue, Net

In June 2022, AMX0035 received marketing authorization with conditions as ALBRIOZA by Health Canada for the treatment of ALS, and we began commercially selling ALBRIOZA within Canada in July 2022. In September 2022, AMX0035 received regulatory approval as RELYVRIO by the FDA for the treatment of ALS, and we launched RELYVRIO in the U.S. in October 2022. All product revenue net, recognized during the period relates to units of ALBRIOZA and RELYVRIO sold in Canada and the U.S., respectively.

Operating Expenses

Cost of Sales

Cost of sales consists primarily of costs associated with the manufacturing of RELYVRIO, ALBRIOZA and certain period costs, which include:

- Direct materials costs;
- Packaging services;
- Transportation costs;
- Manufacturing overhead costs; and
- Royalties related to grants provided to us for the purpose of furthering the research and development of AMX0035 as a therapeutic benefit for ALS. For additional information refer to Note 13 in the Condensed Consolidated Financial Statements included in Part I, Item 1 of this Quarterly Report;

As a result of global macroeconomic conditions, we may experience some disruption and volatility in our global supply chain network, and we may in the future experience disruptions in availability and delays in shipments of raw materials and packaging, as well as related cost inflation.

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 drug product for our preclinical studies and clinical trials, including manufacturing registration and validation batches, as well as pre-commercial manufacturing activities;

- 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 continue to increase in connection with our planned clinical development activities in the near term and in the future and to fund commercialization activities in the U.S., Canada and any other jurisdictions in which AMX0035 is approved. 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 from clinical trials and preclinical studies 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 preclinical and clinical development activities;
- the number and scope of preclinical and clinical trials for separate indications we decide to pursue;
- raising additional funds, if necessary;
- 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 FDA or the EMA, or any other comparable foreign regulatory authority;
- the successful implementation and compliance with the terms of regulatory approvals from applicable regulatory authorities, including our marketing authorization with conditions from Health Canada for ALBRIOZA and the post-marketing requirements from the FDA for RELYVRIO;
- the successful receipt and related terms of regulatory approval from our marketing authorisation application, or MAA, pending with the EMA for ALBRIOZA for the treatment of ALS;
- 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;
- our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the U.S. and internationally;
- our ability to protect our rights in our intellectual property portfolio;
- the commercialization in Canada and the U.S. of AMX0035 (known as ALBRIOZA in Canada and RELYVRIO in the U.S.) and in other potential jurisdictions, 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 in pre-approval market access programs or in commercial access following approval.

A change in the outcome of any of these variables with respect to the development of AMX0035 or any future product candidates could have a significant impact on the cost and timing associated with the development of our product candidates. We may never succeed in obtaining or maintaining, as applicable, regulatory approval for AMX0035 or any future product candidates.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of salaries and related costs for personnel in executive, finance, sales, marketing, as well as administrative functions. Selling, 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; sales and marketing expenses; information technology; facility-related and other operating costs. We anticipate that our selling, general and administrative expenses will continue to increase in the future as we further increase our headcount to support our continued research activities and development of AMX0035 and as we commercialize around the globe. We also anticipate that we will continue to 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. We have received marketing authorization with conditions for ALBRIOZA for the treatment of ALS in Canada and marketing authorization for RELYVRIO for the treatment of ALS in adults in the U.S. and are pursuing regulatory approval of ALBRIOZA for the treatment of ALS in the EU.

Other Income, Net

Interest Income

Interest income consists primarily of the amortization of premiums and accretion of discounts on our short-term investments, and interest income earned on our cash, cash equivalents and short-term investments.

Other Expense, Net

Other expense, net consists primarily of net realized and unrealized losses on foreign exchange transactions.

Income Taxes

Income taxes are determined 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 tax attribute carryforwards using enacted tax rates expected to be in effect in the years in which the differences reverse. Realization of our deferred tax assets is dependent upon the generation of future taxable income, the amount and timing of which are uncertain. Valuation allowances are provided, if, based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. As of June 30, 2023, a portion of our valuation allowance has been reversed through the annual effective tax rate with respect to amounts we expect to realize through current year taxable income. We continue to maintain a full valuation allowance against all of our deferred tax assets not expected to be realized through current year taxable income based on management's evaluation of all available evidence, including our history of incurring significant losses from operations. Our evaluation of all available evidence also includes consideration of regulatory approvals of and developments related to ALBRIOZA and RELYVRIO, including actual and forecasted revenues generated from the sale of these products. Given the early stage of our product launch, we are uncertain about the timing and amount of future sales. We may release all or a portion of the remaining valuation allowance in the near-term; however, the release of the valuation allowance, as well as the exact timing and the amount of such release, continue to be subject to, among other things, our level of profitability, revenue growth, clinical program progression and expectations regarding future profitability.

Results of Operations

Comparison of the three months ended June 30, 2023 and 2022

The following table summarizes our results of operations for the periods presented (in thousands):

	Three Months Ended June 30,			
	2023	**2022**	**$ Change**	**% Change**
Product revenue, net	$ 98,216	$ —	$ 98,216	*NM
Operating expenses:				
Cost of sales	5,580	—	5,580	*NM
Research and development	29,044	24,259	4,785	20 %
Selling, general and administrative	43,391	29,994	13,397	45 %
Total operating expenses	78,015	54,253	23,762	44 %
Income (loss) from operations	20,201	(54,253)	74,454	(137)%
Other income, net:				
Interest income	3,887	402	3,485	867 %
Other expense, net	(81)	(42)	(39)	93 %
Total other income, net	3,806	360	3,446	957 %
Income (loss) before income taxes	24,007	(53,893)	77,900	(145)%
Provision for income taxes	1,933	174	1,759	1011 %
Net income (loss)	$ 22,074	$ (54,067)	$ 76,141	(141)%

* NM - not meaningful

Product revenue, net

We began commercially selling ALBRIOZA within Canada in July 2022 and RELYVRIO within the U.S. in October 2022. For the three months ended June 30, 2023, we recorded approximately $98.2 million of product revenue, net.

Cost of sales

Cost of sales of $5.6 million for the three months ended June 30, 2023, consisted of costs to procure, manufacture and distribute our marketed products, RELYVRIO and ALBRIOZA, and $0.6 million of royalty expense. In addition, included in cost of sales are costs to manufacture our marketed products, which have been provided to patients at no cost to them through either our interim access program or patient assistance program. We expect these costs to continue into 2024, and to a lesser degree, indefinitely. As the Company expensed inventory costs incurred prior to the receipt of regulatory approvals to research and development expense, $3.8 million of costs related to units recognized as revenue are not included in cost of sales for the three months ended June 30, 2023. We expect cost of sales to increase and gross margin to decrease as we deplete these inventories. We expect to use the remaining pre-commercialization inventory for product sales into 2024.

The following table summarizes our research and development expenses for the three months ended June 30, 2023 and 2022 (in thousands):

	Three Months Ended June 30,			
	2023	**2022**	**$ Change**	**% Change**
AMX0035 – ALS	$ 15,028	$ 15,477	$ (449)	(3)%
AMX0035 – PSP	1,211	7	1,204	17,200 %
Payroll and personnel-related	10,464	6,650	3,814	57 %
Other	2,341	2,125	216	10 %
	$ 29,044	$ 24,259	$ 4,785	20 %

Research and development expenses were $29.0 million for the three months ended June 30, 2023, compared to $24.3 million for the three months ended June 30, 2022. During these periods, most of our research and development expenses were related to the development and clinical trials of AMX0035. The increase was primarily due to a $3.8 million increase in payroll and personnel-related costs, a $1.2 million increase in spending on AMX0035 for the treatment of PSP, and a $0.2 million increase in all other costs, offset by a $0.4 million decrease in spending on AMX0035 for the treatment of ALS. The increase in payroll and personnel-related costs was primarily due to an increase in the number of employees supporting research and development efforts. The increase in

spending on AMX0035 for the treatment of PSP was primarily related to costs to support the initiation of the ORION Phase 3 trial. We expect to increase research and development for AMX0035 in other indications in future periods.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $43.4 million for the three months ended June 30, 2023 compared to $30.0 million for the three months ended June 30, 2022. The increase was primarily due to increases of $7.6 million in payroll and personnel-related costs, including stock-based compensation, $1.6 million in consulting and professional services, and $4.2 million in other expenses. The increase in payroll and personnel-related costs was primarily due to hiring additional personnel in commercial and general and administrative functions to support our growth, as well as ongoing commercialization preparation initiatives in the EU. The increases in consulting and professional services and other expenses were primarily due to an increase in spending for commercial activities, operations as a public company and other expenses.

Other Income, Net

Interest Income

Interest income for the three months ended June 30, 2023 was $3.9 million compared to $0.4 million for the three months ended June 30, 2022. The increase was primarily attributable to favorable interest rates and higher investment balances driven by the proceeds received from our 2022 follow-on offering and cash receipts from sales of AMX0035.

Provision for income taxes

We recorded an income tax provision of $1.9 million and $0.2 million for the three months ended June 30, 2023 and 2022, respectively. The income tax provision for these periods was primarily driven by the estimated annual effective tax rate for the year, as well as discrete income tax benefit of $0.1 million and discrete income tax provision of zero during the three months ended June 30, 2023 and 2022, respectively. The income tax benefit for the three months ended June 30, 2023 includes the release of a portion of our valuation allowance that has been reversed through the annual effective tax rate with respect to amounts expected to be realized through current year taxable income. Forecasts of current year taxable income are significantly impacted by a U.S. income tax law change in effect from January 1, 2022 that requires capitalization and amortization of all research and experimentation costs under Section 174 of the Internal Revenue Code. In the future, Congress may consider legislation that would defer the amortization requirement to later years, possibly with retroactive effect, which could have a material impact on the determination of current year taxable income.

Comparison of the six months ended June 30, 2023 and 2022

The following table summarizes our results of operations for the periods presented (in thousands):

	Six Months Ended June 30,			
	2023	**2022**	**$ Change**	**% Change**
Product revenue, net	$ 169,644	$ —	$ 169,644	*NM
Operating expenses:				
Cost of sales	10,863	—	10,863	*NM
Research and development	53,236	45,723	7,513	16 %
Selling, general and administrative	87,397	56,344	31,053	55 %
Total operating expenses	151,496	102,067	49,429	48 %
Income (loss) from operations	18,148	(102,067)	120,215	(118)%
Other income, net:				
Interest income	7,605	533	7,072	1,327 %
Other expense, net	(343)	(61)	(282)	462 %
Total other income, net	7,262	472	6,790	1,439 %
Income (loss) before income taxes	25,410	(101,595)	127,005	(125)%
Provision for income taxes	1,763	320	1,443	451 %
Net income (loss)	$ 23,647	$ (101,915)	$ 125,562	(123)%

* NM - not meaningful

Product revenue, net

We began commercially selling ALBRIOZA within Canada in July 2022 and RELYVRIO within the U.S. in October 2022. For the six months ended June 30, 2023, we recorded approximately $169.6 million of product revenue, net.

Cost of sales

Cost of sales of $10.9 million for the six months ended June 30, 2023, consisted of costs to procure, manufacture and distribute our marketed products, RELYVRIO and ALBRIOZA, and $3.1 million of royalty expense. In addition, included in cost of sales are costs to manufacture our marketed products, which have been provided to patients at no cost to them through either our interim access program or patient assistance program. We expect these costs to continue into 2024, and to a lesser degree, indefinitely. As the Company expensed inventory costs incurred prior to the receipt of regulatory approvals to research and development expense, $7.0 million of costs related to units recognized as revenue are not included in cost of sales for the six months ended June 30, 2023. We expect cost of sales to increase and gross margin to decrease as we deplete these inventories. We expect to use the remaining pre-commercialization inventory for product sales into 2024.

Research and Development Expenses

The following table summarizes our research and development expenses for the six months ended June 30, 2023 and 2022 (in thousands):

	Six Months Ended June 30,			
	2023	2022	$ Change	% Change
AMX0035 – ALS	$ 28,903	$ 30,682	$ (1,779)	(6)%
AMX0035 – PSP	1,211	38	1,173	3,087%
Payroll and personnel-related	19,398	12,515	6,883	55%
Other	3,724	2,488	1,236	50%
	$ 53,236	$ 45,723	$ 7,513	16%

Research and development expenses were $53.2 million for the six months ended June 30, 2023, compared to $45.7 million for the six months ended June 30, 2022. During these periods, most of our research and development expenses were related to the development and clinical trials of AMX0035. The increase was primarily due to a $6.9 million increase in payroll and personnel-related costs, a $1.2 million increase in spending on AMX0035 for the treatment of PSP, and a $1.2 million increase in all other costs, offset by a $1.8 million decrease in spending on AMX0035 for the treatment of ALS. The increase in payroll and personnel-related costs was primarily due to an increase in the number of employees supporting research and development efforts. The increase in spending on AMX0035 for the treatment of PSP was primarily related to costs to support the initiation of the ORION Phase 3 trial. The increase in other costs were primarily due to an increase in preclinical development activities. The decrease in spending on AMX0035 was primarily related to a decrease in inventory expensed as research and development prior to obtaining regulatory approval. We expect to increase research and development for AMX0035 in other indications in future periods.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $87.4 million for the six months ended June 30, 2023 compared to $56.3 million for the six months ended June 30, 2022. The increase was primarily due to increases of $18.1 million in payroll and personnel-related costs, including stock-based compensation, $4.5 million in consulting and professional services, and $8.4 million in other expenses. The increase in payroll and personnel-related costs was primarily due to hiring additional personnel in commercial and general and administrative functions to support our growth, as well as ongoing commercialization preparation initiatives in the EU. The increases in consulting and professional services and other expenses were primarily due to an increase in spending for commercial activities, operations as a public company and other expenses.

Other Income, Net

Interest Income

Interest income for the six months ended June 30, 2023 was $7.6 million compared to $0.5 million for the six months ended June 30, 2022. The increase was primarily attributable to favorable interest rates and higher investment balances driven by the proceeds received from our 2022 follow-on offering and cash receipts from sales of AMX0035.

Provision for income taxes

We recorded an income tax provision of $1.8 million and $0.3 million for the six months ended June 30, 2023 and 2022, respectively. The income tax provision for these periods was primarily driven by the estimated annual effective tax rate for the year, as well as discrete income tax benefit of $0.4 million and discrete income tax provision of zero in the six months ended June 30, 2023 and 2022, respectively. The income tax benefit for the six months ended June 30, 2023 includes the release of a portion of our valuation allowance that has been reversed through the annual effective tax rate with respect to amounts expected to be realized through current year taxable income. Forecasts of current year taxable income are significantly impacted by a U.S. income tax law change in effect from January 1, 2022 that requires capitalization and amortization of all research and experimentation costs under Section 174 of the Internal Revenue Code. In the future, Congress may consider legislation that would defer the amortization requirement to later years, possibly with retroactive effect, which could have a material impact on the determination of current year taxable income.

Liquidity and Capital Resources

Sources of Liquidity

In the second half of 2022, we began generating revenue from the sale of our approved drug product RELYVRIO, known as ALBRIOZA in Canada. To date, we have financed our operations primarily through revenue from the sale of our approved products, the sale and issuance of common stock, convertible preferred stock, convertible notes and grant agreements with the Grantors. As of June 30, 2023, we had cash, cash equivalents and short-term investments of $357.3 million.

From inception through June 30, 2023, we have raised $668.0 million in aggregate proceeds, net of issuance costs, primarily from the issuance of common stock, convertible preferred stock, convertible notes and grant agreements. Based on our current operational plans and assumptions, we believe that the revenue we generate from commercial sales of AMX0035 in the U.S. and Canada and 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 date of the filing of this Quarterly Report.

Capital Resources

We expect our expenses to increase in connection with our ongoing activities, particularly as we advance the preclinical activities, manufacturing and clinical trials of AMX0035 and any future product candidates, execute on our commercialization plans for ALBRIOZA in Canada and RELYVRIO in the U.S., and prepare for the commercial launch of AMX0035 in other jurisdictions, if approved. In addition, 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, our Phase 3 trial of AMX0035 in PSP and our ongoing Phase 2 trial of AMX0035 for the treatment of Wolfram syndrome;
- continue to commercialize AMX0035 (also known as ALBRIOZA in Canada and RELYVRIO in the U.S.) for the treatment of ALS in Canada and the U.S., and pursue launch of ALBRIOZA in Europe, if approved;
- pursue investigational new drug applications of AMX0035 for additional indications;
- conduct preclinical studies and clinical trials for AMX0035 for additional indications and 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 additional marketing approval for AMX0035 or approval for any future product candidates and to support manufacturing on a commercial scale;
- seek additional regulatory approvals for AMX0035 or approvals for any future product candidates that successfully complete clinical trials, if any;
- hire and retain additional personnel, such as preclinical, clinical, quality assurance, regulatory affairs, manufacturing, distribution, legal, compliance, finance, general and administrative, commercial and scientific personnel; and

- develop, maintain, expand and protect our intellectual property portfolio.

In addition, the Sarbanes-Oxley Act of 2002, as well as rules adopted by the SEC and the Nasdaq Global Select Market, requires public companies to implement specified corporate governance practices that are currently not applicable to private companies. Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. 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. Under the statute, we 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 $1.235 billion or more; (ii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iii) the date on which we are deemed to be a large accelerated filer under the rules of the SEC. We will become a large accelerated filer as of December 31, 2023, and as such we will lose emerging growth status on December 31, 2023. To achieve compliance with Section 404 within the prescribed period, we are engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and demands significant effort. 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 believe that the revenue we generate from commercial sales of AMX0035 in the U.S. and Canada and 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 date of the filing of this Quarterly Report. 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.

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 commercialization activities, including manufacturing, marketing, sales and distribution for AMX0035, if approved, in the EU and other territories or for any future product candidates for which we receive regulatory approval;
- the costs, timing and outcome of regulatory review of AMX0035 and any future product candidates;
- our ability to establish and maintain collaborations, marketing, distribution 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 any future outbreak of any highly infectious or contagious diseases;
- the costs of expanding our facilities to accommodate our expected growth in personnel, and the costs of such additional 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 sustain 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 six months ended June 30, 2023 and 2022

The following table summarizes our sources and uses of cash for the periods presented (in thousands):

	Six Months Ended June 30,			
	2023	2022	$ Change	% Change
Net cash provided by (used in) operating activities	$ 3,537	$ (87,096)	$ 90,633	(104)%
Net cash provided by (used in) investing activities	179,216	(94,849)	274,065	(289)%
Net cash provided by financing activities	2,424	199,627	(197,203)	(99)%
Effect of exchange rate changes on cash, cash equivalents and restricted cash equivalents	57	(203)	260	(128)%
Net increase in cash, cash equivalents and restricted cash equivalents	$ 185,234	$ 17,479	$ 167,755	960%

Operating Activities

During the six months ended June 30, 2023, operating activities provided $3.5 million of cash, primarily resulting from our net income of $23.6 million and $17.5 million of non-cash stock-based compensation expense, offset by $4.9 million net accretion of discounts on investments and $33.2 million of net cash used by changes in our operating assets and liabilities.

Net cash used in our operating assets and liabilities primarily consisted of a $32.6 million increase in inventory capitalized during the period and a $18.2 million increase in accounts receivable, net, offset by a $18.5 million increase in accounts payable.

During the six months ended June 30, 2022, operating activities used $87.1 million of cash, primarily resulting from our net loss of $101.9 million, offset by $10.1 million of non-cash stock-based compensation expense and $4.7 million of net cash provided by changes in our operating assets and liabilities.

Net cash used in our operating assets and liabilities primarily consisted of a $4.1 million increase in prepaid expenses and other current assets due to recognition of deferred offering costs related to the initial public offering, and an increase of $0.5 million in other assets, offset by a $3.8 million increase in accounts payable, a $4.8 million increase in accrued expenses and deferred rent due to increased spending for external research and development to support our growth, and a $0.8 million decrease in operating lease right-of use assets.

Investing Activities

During the six months ended June 30, 2023, net cash provided by investing activities was $179.2 million, resulting primarily from $189.5 million of investments that matured, partially offset by $9.8 million of purchases of short-term investments during the period.

During the six months ended June 30, 2022, net cash used in investing activities was $94.8 million, resulting from $1.4 million of purchases of property and equipment and $154.3 million of purchases of short-term investments, offset by $60.9 million of investments that matured during the period.

Financing Activities

During the six months ended June 30, 2023, net cash provided by financing activities was $2.4 million. This amount consisted primarily of $2.6 million of proceeds from exercises of stock options and vesting of stock awards, net of employee taxes paid on these awards.

During the six months ended June 30, 2022, net cash provided by financing activities was $199.6 million. This amount primarily consisted of $216.0 million of gross proceeds from sale of common stock from our initial public offering, offset by $15.1 million of underwriters' discounts and $1.5 million of offering costs paid during the period. Offering costs remaining to be paid as of June 30, 2022 totaled $0.5 million, coupled with offering costs paid in prior periods, results in total net proceeds from the initial public offering of $196.4 million.

Contractual Obligations and Commitments

We enter into agreements in the normal course of business with CMOs for raw material purchases and manufacturing services. As of June 30, 2023, we had committed approximately $86.8 million under these agreements related to raw material purchases and manufacturing services, which are expected to be paid through 2025.

Critical Accounting Policies, Recent Accounting Pronouncements and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the U.S. The preparation of our condensed 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 condensed 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.

There have been no significant changes to our critical accounting policies from those described in "*Management's Discussion and Analysis of Financial Condition and Results of Operations*," disclosed in our 2022 Annual Report.

Emerging Growth Company Status

The Jumpstart Our Business Startups Act of 2012, or 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, for as long as we remain an emerging growth company, 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 earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.235 billion or more, (ii) the last day of our fiscal year following the fifth anniversary of the date of the closing of our initial public offering, (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three year period or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC. We will become a large accelerated filer as of December 31, 2023, and as such we will lose emerging growth status on December 31, 2023.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related to changes in interest rates. As of June 30, 2023 and December 31, 2022, we had cash, cash equivalents and short-term investments of $357.3 million and $346.9 million, respectively. Our cash equivalents are invested primarily in bank deposits and money market mutual funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Our investments are subject to interest rate risk and could fall in value if market interest rates increase. Due to the duration of our investment portfolio and the low risk profile of our investments, we do not believe an immediate 100 basis point change in interest rates would have a material effect on the fair market value of our portfolio. We have the ability to hold our investments until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with vendors that are located outside of the United States. As a result, our operations may be subject to fluctuations in foreign currency exchange rates in the future.

We do not believe that inflation had a material effect on our business, financial condition, or results of operations during the six months ended June 30, 2023 and 2022. However, inflation has had, and may continue to have, an impact on the labor costs we incur to attract and retain qualified personnel.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures.

We maintain “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms and (2) accumulated and communicated to our management, including our principal executive officers and principal financial officer, to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Our management, with the participation of our Chief Executive Officers and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2023. Based on the evaluation, our Chief Executive Officers and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting.

There were no changes in our internal control over financial reporting that occurred during the six months ended June 30, 2023 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have an adverse effect on our business, operating results or financial condition.

Item 1A. Risk Factors.

Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Quarterly Report and in other documents that we file with the SEC, in evaluating our business and our prospects. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and are not the only risks that we face. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of operations.

Summary of Risk Factors

Risks Related to Our Financial Position and Need for Capital

- We have incurred significant losses since our inception and may incur more if we are unable to generate sufficient revenue from our approved products to cover our expenses.
- We have only recently obtained regulatory approval for and launched RELYVRIO in the U.S. and ALBRIOZA in Canada and, prior to their launch, we had never generated revenue from product sales. If revenue from the commercial launches of RELYVRIO in the U.S. and ALBRIOZA in Canada do not cover our expenses, and AMX0035 is not approved in other jurisdictions or for other indications, we may not be profitable.
- We have a limited operating history and our only product, AMX0035, has only recently been approved by the FDA in the U.S. and only recently received marketing authorization with conditions in Canada, which may make it difficult to evaluate the prospects for our future viability.

Risks Related to Commercialization of AMX0035 or Future Product Candidates

- We have limited experience as a commercial company and we may not be successful in commercializing AMX0035 or any future product candidates in the U.S., Canada or anywhere else, if and when approved, and we may be unable to generate meaningful product revenue.
- AMX0035 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 remain profitable.
- If the FDA, Health Canada, the EMA or other 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.
- Healthcare insurance coverage and reimbursement, both from public drug plans and private health care insurers, may be limited or unavailable for RELYVRIO in the U.S. and ALBRIOZA in Canada, and for AMX0035 and any future product candidates, if approved anywhere else, which could make it difficult for us to sell any product candidates or therapies profitably.

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

- We currently depend on the success of AMX0035. If we are unable to maintain, or obtain additional, regulatory approvals for, and successfully commercialize, AMX0035, or experience significant delays in doing so, our business may be materially harmed.

- The delay or denial of regulatory approval, inability to maintain regulatory approval, inability to complete post-marketing requirements, or the requirement to resubmit any marketing application with additional data or information for AMX0035 in any jurisdiction could mean that we need to delay or even cease operations, and a delay in obtaining or inability to maintain such approval would delay commercialization of AMX0035 and adversely impact our ability to generate revenue, our business and our results of operations.
- AMX0035 is a fixed-dose combination drug product and certain regulatory authorities may 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 maintain or obtain regulatory approval for AMX0035 or any future product candidates, our business will be substantially harmed. A finding that our global Phase 3 PHOENIX trial is insufficient to support current or additional marketing authorizations in ALS could lead the FDA or Health Canada to restrict or withdraw prior regulatory approvals for RELYVRIO or ALBRIOZA, respectively, or we could decide, after consultation with regulatory authorities, to voluntarily withdraw RELYVRIO or ALBRIOZA from the marketplace.
- 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 have entered and 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.
- Our use of third parties to manufacture AMX0035 and approved products in compliance with current good manufacturing practices, or cGMP, may increase the risk that we will not have sufficient cGMP-compliant quantities of AMX0035, products, or necessary quantities of such materials on time or at an acceptable cost.

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 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 currently operating in a period of economic uncertainty and capital markets disruption, which has been significantly impacted by geopolitical instability, an ongoing military conflict between Russia and Ukraine, and high inflation and rising interest rates, any of which could have a material adverse effect on our business, financial condition and results of operations.
- We depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.

Risks Related to Our Common Stock

- Unstable market, economic, political and geographical conditions may have serious adverse consequences on our business, financial condition and stock price.

Risks Related to Our Financial Position and Need for Capital

We have incurred significant losses since our inception and may incur more if we are unable to generate sufficient revenue from our approved products to cover our expenses.

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 invested substantial resources into our product development efforts and toward the commercialization of RELYVRIO, which has been recently approved by the FDA, and ALBRIOZA, which has received marketing authorization with conditions from Health Canada, but we have only recently begun generating revenue from product sales to date in the U.S., Canada or elsewhere. We will also continue to incur significant research and development and other expenses related to clinical development, commercialization, approvals in additional jurisdictions and for additional indications, and ongoing operations. 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, preparation for commercialization and, more recently, commercialization activities. Our financial condition and operating results, including our revenues, expenses and net income (loss), 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 may continue to have, an adverse effect on our stockholders' equity and working capital. As of June 30, 2023, we had an accumulated deficit of $330.6 million. We may continue to incur significant losses and our financial results will be highly dependent upon the successful launch and commercial sales of RELYVRIO in the U.S. We will continue to incur expenses related to our research and development activities and pre-commercialization activities in Europe, among other things.

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

- further build out our sales, marketing, pharmacovigilance and distribution infrastructure and scale-up manufacturing capabilities to commercialize AMX0035 and any product candidate for which we may obtain approval;
- continue to develop and conduct clinical trials for AMX0035 for the treatment of ALS, AD, PSP, Wolfram syndrome and potential other additional indications, including our PHOENIX trial, our Phase 3 trial in PSP and our ongoing Phase 2 clinical trial for Wolfram syndrome;
- initiate and continue research, preclinical and clinical development efforts for any future product candidates;
- seek to identify additional product candidates;
- seek to obtain and maintain regulatory approvals in the U.S. and Canada and obtain regulatory approvals in the EU, and other geographies for AMX0035 for the treatment of ALS, AD, Wolfram Syndrome, PSP 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 completion of post-marketing requirements, the potential that the EMA or other regulators require additional data to support the approval of AMX0035 for ALS, failed studies, negative or mixed clinical trial results, safety issues or other regulatory challenges;
- establish sales, marketing, pharmacovigilance, distribution, manufacturing, supply chain and other commercial infrastructure to commercialize any products for which we may in the future obtain regulatory approval;
- 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;
- add equipment and physical infrastructure to support our research and development; and
- acquire or in-license other product candidates and technologies.

We are continuing to build out our infrastructure, including sales and marketing, distribution and manufacturing capabilities, for commercialization of AMX0035 in the U.S. and Canada. As of June 30, 2023, we had 338 full-time employees.

Our expenses could increase beyond our expectations if we are required by the FDA, Health Canada, 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 only recently obtained regulatory approval for and launched RELYVRIO in the U.S. and ALBRIOZA in Canada and, prior to their launch, we had never generated revenue from product sales. If revenue from the commercial launches of RELYVRIO in the U.S. and ALBRIOZA in Canada do not cover our expenses, and AMX0035 is not approved in other jurisdictions or for other indications, we may not be profitable.

Our ability to remain profitable depends on our ability to generate revenue from our approved products, RELYVRIO in the U.S. and ALBRIOZA in Canada. Other than RELYVRIO in the U.S. and ALBRIOZA in Canada, we have not yet launched any other approved products for commercial sale and have only recently begun generating revenue from product sales. Successful commercialization will require achievement of many key milestones, which vary by jurisdiction and may include demonstrating safety and efficacy in clinical trials, obtaining and maintaining 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. Even if we successfully launch and commercialize RELYVRIO in the U.S. and ALBRIOZA in Canada, we may be unable to maintain profitability, unless AMX0035 is approved in other jurisdictions or for additional indications. In June 2023, we announced that the CHMP of the EMA adopted a negative opinion on the application for conditional marketing authorisation of AMX0035 for the treatment of adults with ALS in the EU. Following our request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation. The re-examination procedure is an approximately four-month process, which includes the appointment of a different rapporteur and co-rapporteur from the initial evaluation. At the end of the re-examination, the EMA will review its initial opinion before issuing a final recommendation, which is expected in the fall of 2023. 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 we might sustain 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 sustain profitability on a quarterly or annual basis.

Our failure to 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 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 our only product, AMX0035, has only recently been approved by the FDA in the U.S. and only recently received marketing authorization with conditions in Canada, which may make it difficult to evaluate the prospects for our future viability.

We have only recently commenced our transition from a clinical-stage to a commercial-stage company. Our operations to date have been primarily limited to organizing, staffing and financing our company, raising capital, conducting research and development activities, including preclinical studies and clinical trials and preparing for and commencing commercialization of AMX0035. We have not yet demonstrated an ability to generate significant revenues, or clearly conduct sales and marketing activities necessary for successful product commercialization. In June 2022, AMX0035 received marketing authorization with conditions from Health Canada for the treatment of ALS. One of the conditions of the marketing authorization in Canada is the provision of data from our ongoing PHOENIX trial and other additional planned or ongoing studies. In September 2022, AMX0035 received marketing authorization from the FDA for the treatment of ALS in adults. In June 2023, we announced that the CHMP of the EMA adopted a negative opinion on the application for conditional marketing authorisation of AMX0035 for the treatment of adults with ALS in the EU. Following our request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation. The re-examination procedure is an approximately four-month process, which includes the appointment of a different rapporteur and co-rapporteur from the initial evaluation. At the end of the re-examination, the EMA will review its initial opinion before issuing a final recommendation, which is expected in the fall of 2023. Accordingly, you should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in the early commercial stage, especially pharmaceutical 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 with these activities.

At a second meeting of the FDA's Peripheral and Central Nervous System Drugs Advisory Committee, or the Advisory Committee, on September 7, 2022, relating to AMX0035 for the treatment of ALS, we stated that if our PHOENIX trial is not successful then we will do what is right for patients, which includes voluntarily removing the product from the market. We will work in consultation with regulatory authorities when the PHOENIX data are available. We could also be required by regulatory authorities to withdraw AMX0035 from the marketplace. As part of our approval of RELYVRIO in the U.S., we have post-marketing requirements to conduct carcinogenicity studies in mice and rats, drug-drug interaction studies in human volunteers, and studies in subjects with kidney or liver impairment. The outcomes of these studies including the PHOENIX trial and any potential withdrawal could have a material adverse effect on our business.

We may not satisfy all of the conditions imposed by Health Canada for marketing authorization of ALBRIOZA for the treatment of ALS. If we fail to do so, we may be subject to additional conditions imposed by Health Canada or we may have to cease commercialization of ALBRIOZA, which may impact our prospect for profitability. We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We are transitioning from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We may require substantial additional funding to meet our financial needs and to pursue our business objectives. If we are unable to raise capital if and 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 commercialize AMX0035 in jurisdictions in which it has received regulatory approval and to continue the clinical development of AMX0035 and the preclinical and clinical development of any future product candidates. If we are unable to obtain and maintain marketing approvals 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 continue to develop AMX0035 and any future product candidates and fund our operations. In addition, other unanticipated costs may arise in the course of our development and commercialization 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, developing and commercializing AMX0035 for the treatment of ALS, AD, Wolfram syndrome, PSP and potential additional indications, as well as any future product candidates we may develop;
- the timing of, and the costs involved in, obtaining and maintaining marketing approvals for AMX0035 for the treatment of ALS, AD, Wolfram syndrome, PSP 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;
- 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 sufficient product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt and maintenance of regulatory approval on a jurisdiction-by-jurisdiction basis, 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 economic challenges caused by the COVID-19 pandemic and economic uncertainty in various global markets due to geopolitical instability and conflict, including the ongoing conflict in Ukraine, the global credit and financial markets have experienced extreme volatility and disruptions, including diminished liquidity and credit availability, declines in consumer confidence, high rates of inflation and rising interest rates, 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 revenue we generate from commercial sales of AMX0035 in the U.S. and Canada and 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 date of this Quarterly Report. 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.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We expect our expenses to continue to increase in connection with our planned operations. Unless and until we can generate a substantial amount of revenue and sustain profitability 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 and commercialization 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, local and international 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.

Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults or non-performance by financial institutions or transactional counterparties, could adversely affect our current and projected business operations and its financial condition and results of operations.

Actual events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank, or SVB, was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation, or the FDIC, as receiver. Similarly, on March 12, 2023, Signature Bank and Silvergate Capital Corp. were each swept into receivership. Although a statement by the Department of the Treasury, the Federal Reserve and the FDIC stated that all depositors of SVB would have access to all of their money after only one business day of closure, including funds held in uninsured deposit accounts, borrowers under credit agreements, letters of credit and certain other financial instruments with SVB, Signature Bank or any other financial institution that is placed into receivership by the FDIC may be unable to access undrawn amounts thereunder. If any of our counterparties to any such instruments were to be placed into receivership, we may be unable to access such funds. In addition, if any parties with whom we conduct business are unable to access funds pursuant to such instruments or lending arrangements with such a financial institution, such parties' ability to pay their obligations to us or to enter into new commercial arrangements requiring additional payments to us could be adversely

affected. In this regard, counterparties to SVB credit agreements and arrangements, and third parties such as beneficiaries of letters of credit (among others), may experience direct impacts from the closure of SVB and uncertainty remains over liquidity concerns in the broader financial services industry. Similar impacts have occurred in the past, such as during the 2008-2010 financial crisis. As of June 30, 2023, we had less than $1.5 million of our cash and cash equivalent and short-term investment balances on deposit with SVB, and also held securities in a sweep account purchased through SVB but managed in segregated custodial accounts by third party asset managers.

Inflation and rapid increases in interest rates have led to a decline in the trading value of previously issued government securities with interest rates below current market interest rates. Although the U.S. Department of Treasury, FDIC and Federal Reserve Board have announced a program to provide up to $25 billion of loans to financial institutions secured by certain of such government securities held by financial institutions to mitigate the risk of potential losses on the sale of such instruments, widespread demands for customer withdrawals or other liquidity needs of financial institutions for immediately liquidity may exceed the capacity of such program. There is no guarantee that the U.S. Department of Treasury, FDIC and Federal Reserve Board will provide access to uninsured funds in the future in the event of the closure of other banks or financial institutions, or that they would do so in a timely fashion.

Although we assess our banking relationships as we believe necessary or appropriate, our access to funding sources and other credit arrangements in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect us, the financial institutions with which we have arrangements directly, or the financial services industry or economy in general. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry. These factors could involve financial institutions or financial services industry companies with which we have financial or business relationships, but could also include factors involving financial markets or the financial services industry generally.

The results of events or concerns that involve one or more of these factors could include a variety of material and adverse impacts on our current and projected business operations and our financial condition and results of operations. These could include, but may not be limited to, the following:

- Delayed access to deposits or other financial assets or the uninsured loss of deposits or other financial assets;
- Loss of access to revolving existing credit facilities or other working capital sources and/or the inability to refund, roll over or extend the maturity of, or enter into new credit facilities or other working capital resources;
- Potential or actual breach of contractual obligations that require us to maintain letters or credit or other credit support arrangements; or
- Termination of cash management arrangements and/or delays in accessing or actual loss of funds subject to cash management arrangements.

In addition, investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our financial and/or contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity and our current and/or projected business operations and financial condition and results of operations.

In addition, any further deterioration in the macroeconomic economy or financial services industry could lead to losses or defaults by parties with whom we conduct business, which in turn, could have a material adverse effect on our current and/or projected business operations and results of operations and financial condition. For example, a party with whom we conduct business may fail to make payments when due, default under their agreements with us, become insolvent or declare bankruptcy. Any bankruptcy or insolvency, or the failure to make payments when due, of any counterparty of ours, or the loss of any significant relationships, could result in material losses to us and may material adverse impacts on our business.

Risks Related to Commercialization of AMX0035 or Future Product Candidates

We have limited experience as a commercial company and we may not be successful in commercializing AMX0035 or any future product candidates in the U.S., Canada or anywhere else, if and when approved, and we may be unable to generate meaningful product revenue.

We recently launched ALBRIOZA in Canada and RELYVRIO in the U.S. and, if approved, we also intend to commercialize AMX0035 in the EU with specialized teams, given the relative rarity of ALS and certain of the other indications we are targeting. We are currently continuing to build the global marketing and sales team for the marketing, sales and distribution of AMX0035 and any future product candidates, if approved. In order to successfully commercialize AMX0035 for the treatment of ALS, AD, Wolfram Syndrome, PSP and other indications, 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, we have recruited and trained a U.S. commercial organization which is expensive and

time-consuming. Factors that may inhibit our efforts to commercialize AMX0035 or any future product candidates, if approved, on our own include:

- the inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability to supply the market with our drug product, including manufacturing or distribution challenges we may face;
- 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 community organizations about the safety or efficacy of AMX0035;
- the lack of complementary or symptomatic 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 government and 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 profitability 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 limited 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 and other neurodegenerative disease medical communities and payors on the benefits of AMX0035 or any future product candidates may require significant resources 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 in any jurisdiction, if we are unable to successfully market our products successfully, we will not be able to generate significant revenues from such products.

If we are unable to expand our marketing, manufacturing 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, pharmacovigilance, manufacturing 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 further product launch. Moreover, we cannot be certain that we will be able to develop this capability successfully. We may enter into collaborations regarding any approved product candidates with other entities to utilize their established marketing and distribution capabilities, however, 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, Wolfram syndrome, PSP 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 of 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 incorrect, and new studies may reduce the estimated incidence or prevalence of these diseases. Estimating market opportunities can be particularly challenging for 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 begin to commercialize RELYVRIO in the U.S., ALBRIOZA in Canada and AMX0035, if approved, in other jurisdictions, and learn more about market dynamics and engage with regulators on additional potential marketing approvals, our view of our products' initial potential market opportunity will become more refined. For example, we are now initially focused primarily on the 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 successfully commercialize 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 the U.S., Canada, 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.

AMX0035 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 remain profitable.

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 add AMX0035 to their patients' treatment regimen, or may cease to add AMX0035 to their patients' treatment regimen. Further, patients often acclimate to the treatment regime they are currently taking and do not want to add additional treatments unless their physicians recommend it. Further, they may be unable to add AMX0035 to their treatment regimen 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. Our ability to proactively educate health care professionals and patients may be limited based on the marketing restrictions in a given jurisdiction, specifically as they relate to the particular labeling approved by the applicable health authority.

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 remain 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 or as a single agent or in combination;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product's convenience, tolerability 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.

Off-label use for the treatment of ALS of PB, which is available as a generic drug, along with the potential sale in some jurisdictions of TURSO expose us to additional risks that could reduce or eliminate the commercial opportunity for AMX0035.

We are developing and advancing 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 without approval for the treatment of ALS in some jurisdictions, including the U.S. 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 approved, 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 RELYVRIO in the U.S., ALBRIOZA in Canada and AMX0035, if approved in other jurisdictions, and/or public perception of AMX0035 in the U.S. or abroad.

If the FDA, Health Canada, the EMA or other 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 U.S., 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 U.S. 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 approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or 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.

We have received NCE exclusivity from the FDA for RELYVRIO and such exclusivity expires in September 2027. In addition, in connection with our Health Canada marketing authorization with conditions, ALBRIOZA was added to the Register of Innovative Drugs, which provides an eight year period of market exclusivity. The regulatory authorities in Europe may reach different conclusions from the FDA or Health Canada with respect to exclusivity for AMX0035.

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 determined by the FDA to be 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 for future uses or if current and future candidates are 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 U.S. 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 U.S. of such drug. AMX0035 has been granted orphan drug designation for the treatment of ALS, and with the approval of AMX0035 (RELYVRIO) by the FDA in September 2022 for the treatment of ALS in adults, the product is entitled to orphan drug exclusivity and the FDA cannot 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. This period runs concurrent with the NCE exclusivity period.

Canada’s data protection regime provides an eight year period of market exclusivity for “innovative drugs”, which is independent from patent protection. An innovative drug is a drug that contains a medicinal ingredient not previously approved by Health Canada and that is not a variation of a previously approved medicinal ingredient such as a salt, ester, enantiomer, solvate or polymorph. If a drug qualifies as an “innovative drug” in Canada, generic/and manufacturers are not permitted to seek approval for their product on the basis of a direct or indirect comparison to an innovative drug for the first six years of the data protection period, and Health Canada cannot issue a Notice of Compliance (NOC or marketing approval) for eight years. One of the components of ALBRIOZA (ursodoxicoltaurine) is an innovative drug, and therefore ALBRIOZA was added to the Register of Innovative Drugs upon its approval. The data protection period for ALBRIOZA runs until June 10, 2030 which is eight years from the date its NOC was issued.

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, or NAS, 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. We have applied for NAS status for AMX0035 in the EU. Irrespective of the NAS status, we expect that AMX0035 will be eligible for orphan market exclusivity if the orphan designation is maintained upon grant of a marketing authorisation in the EU. The current orphan medicines regime in the EU entitles an orphan medicine to a 10-year period of market exclusivity, which can be extended to 12 years if the sponsor complies with an agreed upon paediatric investigation plan. However, the European Commission introduced a legislative proposal in April 2023 that, if implemented, could reduce the current exclusivity period for certain orphan medicines to 9 years (or 5 years for well-established use orphan medicines).

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.

AMX0035 or any future product candidates for which we, or any future collaborators, obtain regulatory approval will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. AMX0035 and any future product candidates, if approved, could be subject to post-marketing restrictions, requirements 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 received approval by the FDA for the treatment of ALS in adults (known as RELYVRIO) in September 2022 and marketing authorization with conditions by Health Canada for the treatment of ALS (known as ALBRIOZA) in June 2022. Following our request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation of AMX0035 for the treatment of ALS (known as ALBRIOZA) in the EU and we may seek approval of AMX0035 in additional jurisdictions and in additional indications. 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 U.S. 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 U.S. 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, as part of our approval of RELYVRIO in the U.S., we have post-marketing requirements to conduct carcinogenicity studies in mice and rats, drug-drug interaction studies in human volunteers, and studies in subjects with kidney or liver impairment. Additionally, 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 U.S. Following the approval of our New Drug Submission, or NDS with conditions, Health Canada requires that we submit a Risk Management Plan, or RMP. Health Canada may, as part of the RMP, require that we conduct additional clinical trials. For example, one of the conditions of the marketing authorization in Canada of AMX0035 (ALBRIOZA) is the provision of data from our ongoing PHOENIX trial and other additional planned or ongoing studies. Standard pharmacovigilance activities are also required for any marketed drug product. Any labelling changes or changes in the product supply chain would require a submission to Health Canada for approval before the change may be implemented. Our advertising may be scrutinized by competitors or by health care providers, and complaints could be made to Health Canada or other agencies. Reimbursement in Canada is complex and requires 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 price regulation by the Patented Medicine Prices Review Board, or the 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.

If we are not able to comply with post-approval regulatory requirements, we could have the marketing approvals for AMX0035 or any future approved products withdrawn or restricted by regulatory authorities, or we may voluntarily do so, and our ability to market AMX0035 or any future approved products, to develop AMX0035 in the U.S., Canada or additional jurisdictions or for additional indications, and to develop and seek approval for additional product candidates could become limited, which could adversely affect our ability to achieve or sustain profitability. As a result, the cost of compliance with post-approval regulatory requirements may have a negative effect on our operating results and financial condition.

Healthcare insurance coverage and reimbursement, both from public drug plans and private health care insurers, may be limited or unavailable for RELYVRIO in the U.S. and ALBRIOZA in Canada, and for AMX0035 and any future product candidates, if approved anywhere else, 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 the 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 payors do not adopt such models, our business, financial condition, results of operations and prospects could be adversely affected. For additional information on coverage and reimbursement, see the section entitled "*Business—Government Regulation—Coverage and Reimbursement*" in our 2022 Annual Report.

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 reimbursement amount. Coverage and reimbursement by a third-party payor may depend upon a number of factors.

In the U.S., 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, which uncertainty may be heightened where the product is subject to post-marketing conditions or requirements to provide additional clinical data. In the U.S., 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 U.S. 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 U.S., 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. We may also incur additional challenges when seeking reimbursement from public and private payers where AMX0035 or any future product candidate has been approved subject to post-marketing conditions. 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 there are agreed-upon pricing and reimbursement rates. Prior to these negotiations, a review by CADTH and INESSS are conducted to assess the value that a medicine will provide to the health system. For patented medicines, the PMPRB has jurisdiction over the price at which the medicine is sold, and PMPRB's assessment of an acceptable price can impact negotiations with payors. Such negotiations 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 U.S. 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 U.S., the reimbursement for AMX0035 and any future product candidates we may develop may be reduced compared with the U.S. 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 sustain profitability may be greater. While we have received a positive response from some providers in Canada following Health Canada's approval with conditions of AMX0035 for the treatment of ALS, there is significant uncertainty related to insurance coverage and reimbursement. 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 U.S. 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 U.S. 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. For more information, see the section entitled "*Business – Government Regulation – Current and Future U.S. Healthcare Reform Legislation*" in our 2022 Annual Report.

In the U.S., there have been and continue to be a number of legislative initiatives to contain healthcare costs. The Inflation Reduction Act of 2022, or IRA includes several provisions that may impact our business to varying degrees, including provisions that reduce the out-of-pocket spending cap for Medicare Part D beneficiaries from $7,050 to $2,000 starting in 2025, thereby effectively eliminating the coverage gap; impose new manufacturer financial liability on certain drugs under Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D price caps for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation; and delay until January 1, 2032 the implementation of the HHS rebate rule that would have limited the fees that pharmacy benefit managers can charge. Further, under

the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan drug designation and for which the only approved indication is for that disease or condition. If a product receives multiple orphan drug designations or has multiple approved indications, it may not qualify for the orphan drug exemption. The implementation of the IRA is currently subject to ongoing litigation that challenges the constitutionality of the IRA's Medicare drug price negotiation program. The effects of the IRA on our business and the healthcare industry in general is not yet known.

In addition, President Biden has issued multiple executive orders that have sought to reduce prescription drug costs. In February 2023, HHS also issued a proposal in response to an October 2022 executive order from President Biden that includes a proposed prescription drug pricing model that will test whether targeted Medicare payment adjustments will sufficiently incentivize manufacturers to complete confirmatory trials for drugs approved through FDA's accelerated approval pathway. Although a number of these and other proposed measures may require authorization through additional legislation to become effective, and the Biden administration may reverse or otherwise change these measures, both the Biden administration and Congress have indicated that they will continue to seek new legislative measures to control drug costs.

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. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, sustain profitability or commercialize our product candidates.

Moreover, increasing efforts by governmental and third-party payors in the U.S. and abroad 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 our product candidates. There has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted 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. The effect of these reform efforts on our business and the healthcare industry in general is not yet known.

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.

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.

Governments outside the U.S. 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 trial or other trials 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, physicians, patients 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 U.S. 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. For more information, see the section entitled "*Business – Government Regulation - Other U.S. Healthcare Laws*" in our 2022 Annual Report.

In the U.S., to help patients afford our approved product, we may implement programs to assist them or support third party programs, including patient assistance programs and co-pay coupon programs for eligible patients. Government enforcement agencies have shown increased interest in pharmaceutical companies' product and patient assistance programs, including reimbursement support services, and a number of investigations into these programs have resulted in significant civil and criminal settlements. In addition, at least one insurer has directed its network pharmacies to no longer accept co-pay coupons for certain specialty drugs the insurer identified. Our co-pay coupon programs could become the target of similar insurer actions. In September 2014, the HHS Office of Inspector General, or OIG, issued a Special Advisory Bulletin warning manufacturers that they may be subject to sanctions under the federal anti-kickback statute and/or civil monetary penalty laws if they do not take appropriate steps to exclude Part D beneficiaries from using co-pay coupons. Accordingly, companies exclude these Part D beneficiaries from using co-pay coupons and the same is true for our Amylyx Care Team. It is possible that changes in insurer policies regarding co-pay coupons and/or the introduction and enactment of new legislation or regulatory action could restrict or otherwise negatively affect these patient support programs, which could result in fewer patients using affected products, such as RELYVRIO in the U.S., and therefore could have a material adverse effect on our sales, business, and financial condition.

The distribution of pharmaceutical products is also 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. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform. 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 successfully defend 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 comply 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.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We participate in the Medicaid Drug Rebate Program, the 340B program, the U.S. Department of Veterans Affairs, Federal Supply Schedule, or FSS, pricing program, and the Tricare Retail Pharmacy program, which require us to disclose average manufacturer pricing, and, in the future may require us to report the average sales price for certain of our drugs to the Medicare program. Pricing and rebate calculations vary across products and programs, are complex, and are often subject to interpretation by us, governmental or regulatory agencies and the courts. Furthermore, regulatory and legislative changes, and judicial rulings relating to these programs and policies (including coverage expansion), have increased and will continue to increase our costs and the complexity of compliance,

have been and will continue to be time-consuming to implement, and could have a material adverse effect on our results of operations, particularly if CMS or another agency challenges the approach we take in our implementation. For example, in the case of our Medicaid pricing data, if we become aware that our reporting for a prior quarter was incorrect or has changed as a result of recalculation of the pricing data, we are generally obligated to resubmit the corrected data for up to three years after those data originally were due. Such restatements increase our costs and could result in an overage or underage in our rebate liability for past quarters. Price recalculations also may affect the ceiling price at which we are required to offer our products under the 340B program and give rise to an obligation to refund entities participating in the 340B program for overcharges during past quarters impacted by a price recalculation.

Civil monetary penalties can be applied if we are found to have knowingly submitted any false price or product information to the government, if we are found to have made a misrepresentation in the reporting of our average sales price, if we fail to submit the required price data on a timely basis, or if we are found to have charged 340B covered entities more than the statutorily mandated ceiling price. Additionally, our agreement to participate in the 340B program or our Medicaid drug rebate agreement could be terminated, in which case federal payments may not be available under Medicaid or Medicare Part D for our covered outpatient drugs. Additionally, if we overcharge the government in connection with our arrangements with FSS or Tricare Retail Pharmacy, we are required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges can result in allegations against us under the FCA and other laws and regulations. Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Further, legislation may be introduced that, if passed, would, among other things, further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting, and any additional future changes to the definition of average manufacturer price or the Medicaid rebate amount could affect our 340B ceiling price calculations and negatively impact our results of operations. Additionally, certain pharmaceutical manufacturers are involved in ongoing litigation regarding contract pharmacy arrangements under the 340B program. The outcome of those judicial proceedings and the potential impact on the way in which manufacturers extend discounts to covered entities through contract pharmacies remain uncertain.

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 information processing 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 European Economic Area, or, EEA, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, and similarly, processing of personal data regarding individuals in the United Kingdom, or UK, including personal health data, is subject to the UK General Data Protection Regulation and the UK Data Protection Act 2018, collectively the UK GDPR, and together with the EU GDPR "GDPR"). 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 the consent of the individuals to whom the personal data relates, providing detailed 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 EEA/UK that are not considered by the European Commission and the UK government as providing "adequate" protection to personal data, including the U.S., 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 U.S. Such transfers of personal data outside of the EEA and UK are prohibited unless a valid GDPR transfer mechanism (for example, the European Commission approved Standard Contractual Clauses, or SCCs, and the UK International Data Transfer Agreement/Addendum, or UK IDTA) has been put in place. Where relying on the SCCs /UK IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The international transfer obligations under the EEA/UK data protection regimes will require significant effort and cost, and may result in us needing to make strategic considerations around where EEA/UK personal data is transferred and which service providers we can utilize for the processing of EEA/UK personal data. The GDPR also permits data protection authorities to require the 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 (£17.5 million), 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, EU member states have adopted national laws to supplement the EU GDPR, which may partially deviate from the EU GDPR, and the competent authorities in the EU Member States may interpret EU

GDPR obligations slightly differently from country to country, such that we do not expect to operate in a uniform legal landscape in the EEA with respect to data protection regulations. Although the UK is regarded as a third country under the EU GDPR, the European Commission has now issued a decision recognizing the UK as providing adequate protection under the EU GDPR, or Adequacy Decision, and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted. The UK Government has introduced a Data Protection and Digital Information Bill, or UK Bill, into the UK legislative process to reform the UK's data protection regime, and if passed, the final version of the UK Bill may have the effect of further altering the similarities between the UK and EEA data protection regimes and threaten the UK Adequacy Decision from the European Commission, which may lead to additional compliance costs for us and could increase our overall risk. The respective provisions and enforcement of the EU GDPR and UK GDPR may further diverge in the future and create additional regulatory challenges and uncertainties.

Similar legal requirements are either in place or are being proposed in the U.S. 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 are all aggressive in reviewing consumers' privacy and data security protections. 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 and which was recently amended by the California Privacy Rights Act —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, or the Common Rule, it does apply to other personal information that we may otherwise handle, such as personal information collected in a business to business context and personal information collected from employees, applicants and retirees residing in California. Similar broad consumer privacy laws have already been passed in numerous states, and laws in Virginia, Colorado and Connecticut already have entered into force. In addition, bills for broad consumer privacy laws are being considered in numerous other states and at the federal level.

Compliance with the above requirements and any other data privacy and data security laws and regulations is a rigorous and time-intensive process and requires significant resources and an ongoing 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. 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 the Discovery and Development of Our Current and Future Product Candidates

We currently depend on the success of AMX0035. If we are unable to maintain, or obtain additional, regulatory approvals for, and successfully commercialize, AMX0035, or experience significant delays in doing so, our business may be materially harmed.

We currently only have one advanced product candidate, AMX0035, and our current business and future success depends entirely on our ability to develop, maintain, or obtain additional, regulatory approvals for, and then successfully commercialize, AMX0035, which we are developing for patients with ALS, Wolfram syndrome, AD, PSP and other neurological diseases. 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 are conducting a global Phase 3 clinical trial of AMX0035 in ALS, a Phase 2 clinical trial of AMX0035 in Wolfram syndrome, and intend to conduct additional clinical trials for other indications in the future, including a Phase 3 trial in PSP. 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 recently received approval from the FDA for RELYVRIO for the treatment of ALS in adults and marketing authorization with conditions from Health Canada for ALBRIOZA for the treatment of ALS and following our request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation for ALBRIOZA for the treatment of ALS in the EU. Accordingly, we are investing the majority of our efforts and financial resources in the further development and commercialization of our product candidate, AMX0035, for the treatment of ALS and other diseases. Successful continued development and additional regulatory approvals of AMX0035 for our initial and potential additional indications is critical to the future success of our business. We will need to have sufficient funds for, and successfully enroll and complete, our clinical development of AMX0035 for the treatment of ALS, AD, Wolfram Syndrome, PSP and other indications. The future regulatory and commercial success of AMX0035 or any future product candidates are 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;
- the interpretation of our preclinical and clinical data by regulatory authorities to support marketing approvals;
- potential unforeseen safety issues or adverse side effects;
- receipt and maintenance of marketing approvals from applicable regulatory authorities, including with any expected NCE 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, including of RELYVRIO in the U.S., ALBRIOZA in Canada and AMX0035, if and when approved in other jurisdictions, whether alone or in collaboration with others;
- successfully launching and conducting commercial sales of RELYVRIO in the U.S., ALBRIOZA in Canada and AMX0035 or any future product candidates, if and when approved in other jurisdictions;
- 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, obtain or maintain additional regulatory approvals 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 marketing applications to regulatory authorities, 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 the 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 in any jurisdiction. If we or any of our future collaborators are unable to develop, maintain, or obtain additional, regulatory approvals 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 delay or denial of regulatory approval, inability to maintain regulatory approval, inability to complete post-marketing requirements, or the requirement to resubmit any marketing application with additional data or information for AMX0035 in any jurisdiction could mean that we need to delay or even cease operations, and a delay in obtaining or inability to maintain 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. The research, testing, manufacturing, labeling, approval, sale, marketing and distribution of drug products are subject to extensive regulation by the FDA, Health Canada, the EMA, and other regulatory agencies in the U.S. and other countries, and such regulations differ from country to country. We are not permitted to market AMX0035 until we receive approval or marketing authorization from the relevant regulatory authority. In September 2022, we received approval from the FDA for AMX0035 (RELYVRIO) for the treatment of ALS in adults and, as part of our approval of RELYVRIO in the U.S., we have post-marketing requirements to conduct carcinogenicity studies in mice and rats, drug-drug interaction studies, and studies in patients with kidney or liver impairment. We also received marketing authorization with conditions from Health Canada for AMX0035 (ALBRIOZA) for the treatment of ALS. One of the conditions of the approval in Canada is the provision of data from our ongoing PHOENIX trial and additional planned or ongoing studies. We are also actively pursuing regulatory approval of AMX0035 (ALBRIOZA) for the treatment of ALS in the EU and in June 2023, we announced that the CHMP of the EMA adopted a negative opinion on the application for conditional marketing authorisation. Following our request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation. The re-examination procedure is an approximately four-month process, which includes the appointment of a different rapporteur and co-rapporteur from the initial evaluation. At the end of the re-examination, the EMA will review its initial opinion before issuing a final recommendation, which is expected in the fall of 2023. It is possible that we may be unable to successfully achieve EMA approval from the re-examination.

The FDA, Health Canada, the EMA or any other foreign regulatory agency can delay, limit, deny or withdraw approval to market AMX0035 for many reasons, including:

- our inability to demonstrate to the satisfaction of, the FDA, Health Canada, 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;
- the FDA's, Health Canada's, the EMA's or any other applicable foreign regulatory agency's disagreement with the interpretation of data from preclinical and clinical studies and trials, such as the FDA's differing interpretations of certain data, including sensitivity and statistical analyses, from our CENTAUR trial and OLE as presented at the meetings of the FDA's Advisory Committee on March 30, 2022 and September 7, 2022 and as considered by the CHMP of the EMA;
- 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;
- the FDA's, Health Canada's, the EMA's or any other applicable foreign regulatory agency's requirement for additional preclinical or clinical studies or trials, including studies to satisfy applicable rules governing fixed dose combination products or post-market requirements;
- the FDA's, Health Canada's, the EMA's or any other applicable foreign regulatory agency's having differing requirements for the trial protocols used in our clinical trials;
- the FDA's, Health Canada's, the EMA's or any other applicable foreign regulatory agency's non-approval of the formulation, labeling and/or the specifications of AMX0035;
- the FDA's, Health Canada'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 the FDA, of Health Canada, 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 the FDA, Health Canada, the EMA or other regulatory approval processes and are commercialized.

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 the FDA, Health Canada, 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.

AMX0035 is a fixed-dose combination drug product and certain regulatory authorities may 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 FDA's 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 preclinical 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 where we intend to seek regulatory approval. In the EU, we have only submitted preclinical data to demonstrate the clinical effects of each component in AMX0035, PB and TURSO (also known as TUDCA), in our MAA. There can be no assurance that the EMA will conclude that our preclinical data are sufficient for these purposes or, even if they are, that the results from our preclinical studies demonstrate the clinical effects of each component in AMX0035 for the treatment of ALS. We may be required to produce clinical data supporting the contribution of each component when present at the levels included in the fixed-dose combination in order to obtain marketing authorization in the EU.

While the FDA has approved AMX0035 (known as RELYVRIO) as a fixed-dose combination product for the treatment of ALS in adults, we may be required by the FDA and comparable foreign regulatory authorities to satisfy the fixed-dose combination rule for AMX0035 or any other fixed-dose combination products we may develop for the treatment of any other indications we may pursue in advance of approval.

If the FDA, the EMA or other comparable foreign regulatory authorities require us to conduct one or more clinical trials to support such a demonstration, 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 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. Historically, efforts by pharmaceutical companies in the field of neurodegenerative and CNS disorders have experienced 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 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 disorders. Our future success is highly dependent on the successful development and commercialization of AMX0035 and any future product candidates for treating neurodegenerative and CNS disorders. Developing and 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 and maintaining 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 maintain or obtain regulatory approval for AMX0035 or any future product candidates, our business will be substantially harmed. A finding that our global Phase 3 PHOENIX trial is insufficient to support current or additional marketing authorizations in ALS could lead the FDA or Health

Canada to restrict or withdraw prior regulatory approvals for RELYVRIO or ALBRIOZA, respectively, or we could decide, after consultation with regulatory authorities, to voluntarily withdraw RELYVRIO or ALBRIOZA from the marketplace.

We, and any future collaborators, are not permitted to commercialize, market, promote or sell any product candidate in the U.S., Canada, or the EU 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, and typically takes many years following the commencement of clinical trials and depends upon numerous factors, including substantial discretion of such 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. The FDA in any approval needs to determine that there is substantial evidence of effectiveness. This finding can be substantiated based on two adequate and well-controlled studies, or in certain circumstances on a single, large, multicenter, adequate and well-controlled study that is very persuasive or from a single adequate and well-controlled study together with confirmatory evidence. FDA regulations and guidance also allow for greater flexibility and tolerance for uncertainty in the context of rare and fatal diseases. In June 2022, we obtained marketing authorization with conditions for AMX0035 (ALBRIOZA) in Canada and in September 2022, we received approval from the FDA for AMX0035 (RELYVRIO) in the U.S. While we have received approval from the FDA and marketing authorization with conditions from Health Canada, and have submitted an MAA to the EMA, to date, we have not submitted any other similar drug approval submissions to comparable foreign regulatory authorities for AMX0035 or any other product candidate. In June 2023, we announced that the CHMP of the EMA adopted a negative opinion on the application for conditional marketing authorisation of AMX0035 (ALBRIOZA), for the treatment of adults with ALS in the EU. Following our request for a formal re-examination in July 2023, CHMP is re-examining its initial opinion on the current application for conditional marketing authorisation. The re-examination procedure is an approximately four-month process, which includes the appointment of a different rapporteur and co-rapporteur from the initial evaluation. At the end of the re-examination, the EMA will review its initial opinion before issuing a final recommendation, which is expected in the fall of 2023.

One of the conditions of the marketing authorization in Canada is the provision of data from our ongoing PHOENIX trial. There is no guarantee that Health Canada will accept the data from our PHOENIX trial as satisfying the conditions and grant a marketing authorization without conditions for ALBRIOZA for the treatment of ALS. Health Canada could require us to make further undertakings with respect to confirmatory clinical trials if it is not satisfied with the data from the PHOENIX trial, in order for AMX0035 to continue to be marketed in Canada.

Our approval of RELYVRIO by the FDA was granted following a positive recommendation for approval at the second virtual meeting of the Advisory Committee held on September 7, 2022. The Advisory Committee initially met on March 30, 2022 and voted 4 (yes) and 6 (no) on the question of whether the data from our randomized, controlled Phase 2 CENTAUR trial and OLE established a conclusion that AMX0035 is effective in the treatment of patients with ALS. At the second meeting of the Advisory Committee, the Advisory Committee voted 7 (yes) to 2 (no) in response to the question of whether available evidence of effectiveness is sufficient to support approval of AMX0035 for the treatment of ALS, taking into account the unmet need in ALS, the status of the ongoing PHOENIX trial and the seriousness of ALS. Although the FDA subsequently approved RELYVRIO for the treatment of ALS in adults, at this meeting and the previous Advisory Committee meeting, the FDA presented concerns regarding choices of statistical models for the prespecified primarily analysis and the interpretability of the survival results included in our marketing application. Other regulatory authorities may present similar concerns regarding our data when reviewed to support marketing applications for AMX0035 for the treatment of ALS. For example, the CHMP of the EMA adopted a negative opinion on the application for conditional marketing authorisation of AMX0035 for the treatment of adults with ALS in the EU relating to the sufficiency of the clinical data in CENTAUR to support approval. If we experience delays in obtaining and maintaining regulatory approval or if we fail to obtain or maintain such approvals, the commercial prospects for AMX0035 may be harmed and our ability to generate revenues or obtain additional approvals and the value of our common stock will be materially impaired.

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 could fail to obtain additional regulatory approvals, and any of our future product candidates could fail to obtain regulatory approvals, for many reasons, including the following:

- the FDA, Health Canada, the EMA or other comparable foreign regulatory authorities may disagree as to the design or implementation of our clinical trials and may require additional data to support regulatory approval;
- 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(s) 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, Health Canada, the EMA and comparable authorities in other countries may disagree with our interpretation of data from clinical trials or preclinical studies and our request may require additional trials or studies to support marketing approval;
- 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 other comparable foreign regulatory authority to obtain regulatory approval in the U.S., Canada, 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 past or 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.

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. This finding can be substantiated based on two adequate and well-controlled studies, or in certain circumstances on a single, large, multicenter, adequate and well-controlled study that is very persuasive or from a single adequate and well-controlled study together with confirmatory evidence. The FDA may not agree that this standard is met. Accordingly, there can be no assurance that for AMX0035 or future product candidates the FDA and other regulatory agencies, including Health Canada and the EMA, will not require additional clinical trials beyond what we may plan to conduct. 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 post-marketing requirements from the respective agency. In September 2022, we received approval for AMX0035 from the FDA for the treatment of ALS in adults, and as a part of our approval, we have post-marketing requirements to conduct carcinogenicity studies in mice and rats, drug-drug interaction studies, and studies in patients with kidney or liver impairment. In July 2022, we received marketing authorization for AMX0035 from Health Canada, with conditions, for the treatment of ALS. One of the conditions of the approval is the provision of data from our ongoing PHOENIX trial. There is no guarantee that Health Canada will accept the data from our PHOENIX trial and grant authorization without conditions for AMX0035 for the treatment of ALS. Additionally, 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 or after the review period for these applications, is not sufficient to support our request for marketing authorization in the EU. It is typically the case not just in the U.S., but also in Canada and Europe, that marketing approvals are based on two Phase 3 clinical studies. Moreover, any finding by another regulatory authority that our global Phase 3 PHOENIX trial is insufficient to support

additional marketing authorizations in ALS could lead the FDA or Health Canada to restrict or withdraw prior regulatory approvals for RELYVRIO and ALBRIOZA, respectively. At the second meeting of the Advisory Committee on September 7, 2022, we stated that if our PHOENIX trial is not successful then we will do what is right for patients, which includes voluntarily removing the product from the market. Any such findings by a regulatory authority or decision to voluntarily withdraw AMX0035 from the marketplace would materially harm our ability to generate revenue and remain profitable.

In addition, disruptions caused by any future public health crisis 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 in the event of a future public health crisis. 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 such future highly infectious or contagious diseases, 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 any future outbreak of any highly infectious or contagious diseases. As a result of a future public health crisis, we may face delays in meeting our anticipated timelines for our ongoing and planned clinical trials.

In addition, regulatory authorities may subject our clinical or manufacturing operations to inspections, including routine surveillance, bioresearch monitoring and pre-approval inspections. For example, with respect to new sites or facilities in the EEA which have never had a 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 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 preclinical studies and 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 regulatory approval to commercialize 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. Preclinical and clinical testing are expensive and can take many years to complete, and their 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, which could impact our ability to obtain additional regulatory approvals for AMX0035, to satisfy any applicable post-market conditions or requirements or to continue marketing AMX0035 in the U.S. and Canada. We could also be required by regulatory authorities to withdraw AMX0035 from the marketplace. This could impact our development plans for AMX0035 for other indications and future product candidates and could impact our results of operations.

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 delay marketing approvals or 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 or maintain any prior-issued regulatory approval for AMX0035 for ALS (including our FDA approval and our marketing authorization with conditions from Health Canada) 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, Wolfram Syndrome, PSP or potential additional indications and to demonstrate the efficacy and safety of AMX0035, including each component thereof, necessary to obtain and maintain regulatory approval to market AMX0035, including if our global Phase 3 PHOENIX trial is not successful, would significantly harm our business and ability to continue developing and marketing AMX0035 for any indications. Our product candidate development costs will also increase if we experience delays in testing or obtaining and maintaining 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 or the need for additional data from our clinical trials 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 or maintaining approvals for

the commercialization of AMX0035 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 its development and commercialization, including its 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 U.S. and in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing that product candidate in a given jurisdiction. Although we have invested substantial time and resources to date in pursuit of regulatory approval and toward potential commercialization, we have only received regulatory approval for AMX0035 (RELYVRIO) in the U.S. and marketing authorization with conditions for AMX0035 (ALBRIOZA) in Canada and have not received any other regulatory approvals 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 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 U.S., 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 during the review process that our data are insufficient for approval and require additional preclinical, clinical or other studies. For example, during the review of our NDA for AMX0035 for the treatment of ALS, the FDA requested clarifying information regarding our preclinical and clinical data and during the Advisory Committee meetings noted certain concerns with interpretation of our clinical data. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit, or prevent marketing approval of, or limit the approved labeling for, a product candidate. For example, while we have conducted preclinical studies in various models of neurodegenerative diseases, it is the view of the FDA that the mechanism by which RELYVRIO exerts its therapeutic effects in patients with ALS is unknown. In addition, in the approved labeling for RELYVRIO, the FDA noted that the post hoc, long-term exploratory survival analysis should be interpreted with caution given the limitations of data collected outside of a controlled study. Additionally, the FDA has discretion to refer an application for a novel drug or a drug that presents difficult questions of safety or efficacy to an advisory committee. For example, our approval of RELYVRIO by the FDA was granted following the second virtual meeting of the Advisory Committee held on September 7, 2022. The Advisory Committee initially met on March 30, 2022 and voted 4 (yes) and 6 (no) on the question of whether the data from our randomized, controlled Phase 2 CENTAUR trial and OLE established a conclusion that AMX0035 is effective in the treatment of patients with ALS. At the second meeting of the Advisory Committee, the Advisory Committee voted 7 (yes) to 2 (no) in response to the question of whether available evidence of effectiveness is sufficient to support approval of AMX0035 for the treatment of ALS, taking into account the unmet need in ALS, the status of the ongoing PHOENIX trial and the seriousness of ALS. At this meeting and the previous Advisory Committee meeting, the FDA presented concerns regarding choices of statistical models for the prespecified primarily analysis and the interpretability of the survival results. Additionally, in July 2022 we received marketing authorization with conditions of AMX0035 (ALBRIOZA) from Health Canada for the treatment of ALS. One of the conditions of the marketing authorization in Canada is the provision of data from our ongoing PHOENIX trial. There is no guarantee that Health Canada will accept the data from our PHOENIX trial as satisfying the conditions and grant a marketing authorization without conditions for ALBRIOZA for the treatment of ALS or that the PHOENIX trial will be successful. Health Canada could require us to make further undertakings with respect to confirmatory clinical trials if it is not satisfied with the data from the PHOENIX trial, in order for AMX0035 to continue to be marketed in Canada. As such, we may be unable to obtain or to maintain the marketing approvals we are pursuing and any marketing approvals we ultimately obtain, including any conditional approvals, may be denied, limited, withdrawn, or subject to restrictions or post-approval commitments that could render the approved product not commercially viable.

If we experience delays in obtaining and maintaining 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 or preliminary 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. In addition, the clinical results seen in the CENTAUR trial may not be repeated in our global Phase 3 PHOENIX clinical trial, which may materially impact our ability to obtain authorization without conditions for ALBRIOZA in Canada, to maintain our approval for RELYVRIO in the U.S., and to continue development of AMX0035 for additional indications or of 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 have in the past utilized and may in the future 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 current or future trials and could result in delays of completion 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 or expanded access to 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, Wolfram syndrome, PSP 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 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 and maintain 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 public health epidemics and related illness, 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. For example, in seeking approval of AMX0035 in Europe, we submitted data supporting a different formulation of AMX0035 from the formulation evaluated in the CENTAUR trial. Changes to commercial formulations from those studied clinically could lead regulatory authorities to delay the approval of our marketing applications until we can demonstrate through additional clinical data that there is comparability in the bioavailability of the two different formulations or may require us to revert to the prior formulation evaluated clinically. Should we have to conduct comparability testing to bridge earlier clinical data obtained from product candidates produced under earlier manufacturing methods or formulations with the planned commercial formulation, regulatory authorities may disagree on the interpretation of results from this testing. This could delay completion of clinical trials, require 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, denial or withdrawal of regulatory approval by the FDA, Health Canada, the EMA or comparable foreign regulatory authorities. Results of our preclinical studies or 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, abdominal pain, nausea, upper respiratory infection, constipation, headache, fatigue, proteinuria, and decreased appetite. In animal studies, administration of AMX0035 to rats throughout pregnancy and lactation resulted in increased offspring mortality at all doses tested, which were less than or similar to the clinical doses tested in our clinical trials. 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 for ALS or other indications. 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 or severe 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 and TURSO has been approved in Italy for diseases of cirrhotic liver disorders such as primary biliary cirrhosis. 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 receives marketing approval and is commercialized in a jurisdiction, we would continue to be subject to the risks that the applicable regulatory authorities could revoke approval of PB or TURSO or any active moiety in AMX0035, if applicable, or that efficacy, manufacturing or supply issues could arise with PB or TURSO 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, Wolfram Syndrome, PSP 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. On March 18, 2022, we launched an FDA-authorized EAP in the U.S. for AMX0035 for certain adults with ALS and this program will be wound down alongside the commercial launch of RELYVRIO in the U.S., with a target close of the EAP in the first half of 2023. We may launch additional EAPs of AMX0035 in the EU.

In the past, media attention to individual patients' expanded access requests has resulted in the introduction and enactment of legislation at the local and national level, including the Accelerating Access to Critical Therapies for ALS Act and prior "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 EAPs and the former of which is intended to support research and development related to ALS, specifically. A possible consequence of both activism and legislation in this area may be the need for us to initiate an EAP beyond that which we have submitted to the FDA or to make AMX0035 or any future product candidates more widely available sooner than anticipated.

In addition, some patients who receive access to drugs prior to their commercial approval through compassionate use, EAPs or right to try access have life-threatening illnesses and have exhausted all other available therapies. The risk for SAEs in this patient population is high, which could have a negative impact on the safety profile of AMX0035 or future product candidates, which could cause significant delays or an inability to successfully commercialize AMX0035 or future product candidates, which could materially harm our business. We may in the future need to restructure or pause any future compassionate use and/or EAP we initiate 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 continue to utilize appropriate social media in connection with our commercialization efforts for RELYVRIO in the U.S. and ALBRIOZA in Canada, and in any other jurisdictions where we obtain regulatory approvals. Social media practices in the biotechnology and pharmaceutical 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 on treatment with AMX0035 or 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 product candidates or indications and modifications for which to investigate AMX0035 in the future. We may expend our limited resources to pursue particular product candidates or indication or formulation for AMX0035 and fail to capitalize on such product candidates or indications or formulations of AMX0035 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 prioritize more difficult. As a result, we may forgo or delay pursuit of opportunities with other indications that we believe could have had greater commercial potential or likelihood of success. In addition, we are continuing to evaluate plans to explore the use of AMX0035 in patients with AD, Wolfram syndrome, PSP and other indications, and other product candidates in ALS and additional neurodegenerative diseases. However, we may focus on or pursue one or more of our target indications over other potential indications and product candidates and such development efforts may not be successful, which would cause us to delay the clinical development and approval of AMX0035, and other product candidates. 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 or formulations of AMX0035 or other product candidates 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. Additionally, Mitsubishi Tanabe Pharma America, Inc., or MTPA, is developing an oral alternative to Radicava. In the first quarter of 2022, the FDA accepted MTPA's application for priority review of its oral alternative to Radicava and in May 2022, the FDA approved its oral alternative to Radicava. Our potential competitors include pharmaceutical and biotechnology companies, such as Biogen, Inc., UCB S.A. and PTC Therapeutics, Inc., specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions. In April 2023, the FDA granted accelerated approval to Biogen, Inc.'s NDA for toferson, an investigational drug for superoxide dismutase 1 (SOD1) ALS.

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.

Following 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 commercialize. Competitive products may make any products we commercialize 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. We expect to face competition with respect to our commercialization of RELYVRIO in the U.S. and ALBRIOZA in Canada and any future product candidates, if approved. Following approval by Health Canada, the FDA or the EMA for the commercial sale of AMX0035 or any future product candidates, 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.

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 though the FDA has approved AMX0035 (RELYVRIO) and Health Canada has granted marketing authorization with conditions of AMX0035 (ALBRIOZA), comparable regulatory authorities in the 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 the U.S., Canada 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 U.S., 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 received approval for AMX0035 (RELYVRIO) in the U.S. and marketing authorization with conditions for AMX0035 (ALBRIOZA) in Canada and have submitted a marketing application in the EU. Regulatory authorities in jurisdictions outside of the U.S. 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 except the U.S. and Canada, and we do not have experience in obtaining regulatory approval in international markets outside of Canada. 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 U.S. and the EU and for the treatment of Wolfram syndrome in the U.S., 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 U.S. 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 U.S., or a patient population of greater than 200,000 people in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the EU, the EMA Committee for Orphan Medicinal Products grants orphan 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 no more than five in 10,000 people in the EU when the application is made. 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 product in the EU would be sufficient to justify the necessary investment in developing the 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 U.S., and with the approval of AMX0035 (RELYVRIO) by the FDA in September 2022 for the treatment of ALS in adults, the product is entitled to orphan drug exclusivity and the FDA cannot 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 addition, in June 2020, the EMA granted orphan medicine 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 U.S. 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 U.S. 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 product no longer meets the criteria for orphan designation or if the product 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. Legislation has been proposed by the European Commission that, if implemented, has the potential in some cases to shorten the ten-year period of orphan marketing exclusivity. 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 Wolfram syndrome, if we receive approval for AMX0035 for a modified or different indication, our current orphan designation 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 though AMX0035 is entitled to orphan drug exclusivity, 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 U.S., or a patient population of greater than 200,000 people in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. 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 U.S. 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 pursue priority review designation for product candidates that we may develop, but we might not receive such designations, and priority review designations may not lead to a faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. For example, we received priority review for AMX0035 for the treatment of ALS, and we may in the future request priority review designation for any future product candidates, however, we cannot assume that any application for future indications of AMX0035 or any other product candidate we may develop will meet the criteria for that designation. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to standard FDA review and approval. For example, the FDA originally set the PDUFA date for AMX0035 for the treatment of ALS, for June 29, 2022, and then extended the review timeline for our NDA to September 2022. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

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.

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. The use of AMX0035 by us and any collaborators in clinical trials, and the sale of AMX0035 in the U.S., Canada and in other jurisdictions, 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 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 as we commercialize AMX0035 in the U.S., Canada and other jurisdictions, if approved, 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 and maintain 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 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 fail to conform to cGMPs and 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, Health Canada, 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, in any jurisdiction where 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 U.S. and will require us to develop and implement costly compliance programs.

We have operations in the U.S. and Canada and expect to engage in operations in other jurisdictions, including the EU, 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 currently or 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 U.S. 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 U.S., 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 U.S., 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 U.S., 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 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 and maintain 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 commercialization of RELYVRIO in the U.S. and ALBRIOZA in Canada and the potential commercialization of AMX0035 in other jurisdictions and of 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 have entered and 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 or to identify novel drug candidates for neurodegenerative diseases as with our partnership with Sunnybrook Research Institute. 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 or maintain regulatory approval or successfully 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 by regulatory authorities. For example, at the Advisory Committee meeting on March 30,

2022, the FDA noted a number of concerns that, in the FDA's view, impacted the interpretability of the results from the CENTAUR trial. 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, the EMA or other regulatory body may require us to perform additional clinical trials before approving AMX0035, including for additional indications, or any future product candidates, which would delay the regulatory approval process. We cannot be certain that, upon inspection, the FDA, the EMA or other regulatory body 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, such as 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 and approved products in compliance with cGMP may increase the risk that we will not have sufficient cGMP-compliant 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 in accordance with applicable law, regulations and standards. 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 for expanded access and commercial supply, 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. Moreover, 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 whether the economic challenges caused by the COVID-19 pandemic continue to impact the global economy and supply chains, among many other factors. 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, including cGMPs, 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, or as a result of economic or political developments, including the ongoing conflict in Ukraine and global economic instability;
- 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 a 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 adversely impact our ability to commercialize AMX0035 in the U.S. and Canada, and 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 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, expanded access and, commercialization of AMX0035 in the U.S. and Canada, and any subsequent commercialization of AMX0035 in other jurisdictions, if approved, 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 and compliance with cGMPs, then we may not be able to meet the demands of clinical trials, expanded access or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operation.

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 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 U.S. 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 sustain profitability. To protect our proprietary position, we have filed patent applications and may file other patent applications in the U.S. 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 U.S. Furthermore, patents have a limited lifespan. In the U.S., 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 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 U.S., 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 U.S. 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 U.S. 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 U.S. 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 U.S. 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 U.S. 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 U.S. 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 U.S. 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 U.S. For example, patent laws in various jurisdictions, including jurisdictions 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 U.S. 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 U.S. for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the U.S. 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 U.S. 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 U.S. 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 U.S., 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 U.S. 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 U.S. 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 U.S. or foreign laws;
- we may not successfully commercialize AMX0035 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, or IT, systems, but it is possible that these security measures could be breached. In addition, courts outside the U.S. 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 U.S., 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 U.S. 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 U.S., 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 U.S. 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 requirements. 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 failure to otherwise satisfy any of the numerous applicable requirements. Moreover, the applicable authorities, including the FDA and the USPTO in the U.S., 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 U.S. and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

The U.S. 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 U.S. 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 U.S. can be less extensive than those in the U.S. 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 U.S. and the EU do not afford intellectual property protection to the same extent as the laws of the U.S. 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 U.S. and the EU or from selling or importing products made from our inventions in and into the U.S. 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 U.S. 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 U.S. 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 U.S., 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 a 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 in turn 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 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 U.S. 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. Moreover, 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 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. For instance, from 2020 through 2022, we experienced certain impacts of the COVID-19 pandemic, including alterations to our preclinical and clinical trial activities, such as scheduling certain work off-site and performing off-site assessments. 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.

Any negative impact 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, which could have a material adverse effect on our financial results. The COVID-19 pandemic and other global macroeconomic factors have also caused significant volatility in public equity markets and disruptions to the U.S. 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 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 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 are currently operating in a period of economic uncertainty and capital markets disruption, which has been significantly impacted by geopolitical instability, an ongoing military conflict between Russia and Ukraine, and high inflation and rising interest rates, any of which could have a material adverse effect on our business, financial condition and results of operations.

U.S. and global markets are experiencing volatility and disruption caused by the ongoing Russia-Ukraine conflict and the effects of sanctions imposed on Russia as a result of the conflict. In February 2022, a full-scale military invasion of Ukraine by Russian troops began. Although the length and impact of the ongoing military conflict is highly unpredictable, the conflict in Ukraine has led to market disruptions, including significant volatility in commodity prices, credit and capital markets, as well as supply chain interruptions, which has contributed to record inflation globally. We are continuing to monitor inflation, the situation in Ukraine and global capital markets and assessing its potential impact on our business, including the impact on the supply chains we rely on for the manufacture of AMX0035 or other future product candidates.

Although, to date, our business has not been materially impacted by the ongoing military conflict between Russian and Ukraine, geopolitical tensions, or record inflation, it is impossible to predict the extent to which our operations will be impacted in the short and long term, or the ways in which such matters may impact our business. The extent and duration of the conflict in Ukraine, geopolitical tensions, record inflation and resulting market disruptions are impossible to predict but could be substantial. Any such disruptions may also magnify the impact of other risks we face.

Inadequate funding for the FDA, the SEC and other government agencies, including from government shutdowns, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, the ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

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 and 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, 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, 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 June 30, 2023, we had 338 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 to 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 marketing, 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 currently expect to continue to significantly increase 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. 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

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 may 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 Quarterly Report, 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 additional regulatory submissions for AMX0035 or 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 approvals 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 successfully 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 and rate of expenditures;
- 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, geographical, and economic conditions, including the impact of the COVID-19 pandemic, historically high inflation, rising interest rates and the ongoing conflict in Ukraine; and

- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and pharmaceutical 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.

Unstable market, economic, political and geographical conditions may have serious adverse consequences on our business, financial condition and stock price.

As widely reported, global credit and financial markets have experienced extreme volatility and disruptions in the past several years, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in the rate of inflation and interest rates, increases in unemployment rates and uncertainty about economic stability, including most recently in connection with the COVID-19 pandemic and the conflict in Ukraine. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. Our business could also be impacted by volatility caused by geopolitical events such as the war in the Ukraine. If the current equity and credit markets deteriorate, or do not improve, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay, scale back or discontinue the development and commercialization of one or more of our product candidates or delay our pursuit of potential in-licenses or acquisitions. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget.

As of December 31, 2023, we will no longer be an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies will no longer apply to us.

We are currently an emerging growth company but because as of June 30, 2023, the market value of our common stock that was held by non-affiliates exceeded $700 million, we will no longer qualify for such status commencing December 31, 2023. As a large-accelerated filer, we will be subject to certain disclosure requirements that are applicable to other public companies that have not been applicable to us as an emerging growth company. These requirements include:

- compliance with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- compliance with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- full disclosure obligations regarding executive compensation; and
- compliance with the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

A significant portion of our total outstanding shares may be sold into the market, 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. As of August 7, 2023, we had outstanding 67,377,743 shares of common stock, which may be resold in the public market immediately without restriction, unless held by our affiliates. Moreover, holders of approximately 18.5 million shares of our common stock 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.

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding common stock, own a significant portion of our common stock. As a result, these stockholders acting together, could be able to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, could significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that our stockholders may feel are in their best interest.

Delaware law and provisions in our amended and restated certificate of incorporation, or our certificate of incorporation, and amended and restated bylaws, or our bylaws, could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Provisions of our certificate of incorporation and bylaws 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 certificate of incorporation and bylaws:

- permit our board of directors to issue up to 10,000,000 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 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 is 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 certificate of incorporation and 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 U.S. 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, or the 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 certificate of incorporation, 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.

We are 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 current fiscal year end. This assessment will need to include disclosure of any material weaknesses identified by our management in our internal controls over financial reporting. However, for as long as we remain an emerging growth company, 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, which we expect to occur as of December 31, 2023 when we become a large accelerated filer. Therefore, on next year's annual report on Form 10-K, we will be required to obtain auditor attestation, and if we then have a material weakness, we will receive an adverse opinion regarding our internal control over financial reporting from our independent registered public accounting firm. We are required to disclose significant changes made in our internal controls procedures on a quarterly basis.

We continue 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 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 necessary to comply with Section 404.

We have identified past 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.

Our bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the U.S. 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 bylaws 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 amended and restated certificate of incorporation or bylaws;
- any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our 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 certificate of incorporation further provides that the federal district courts of the U.S. 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 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 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 our public offerings.

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, 2023 and continuing through and including January 1, 2032, by 5.0% 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, 2023 (through January 1, 2032), by the lesser of (i) 1.0% 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) 1,210,000 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 incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives.

As a public company, we incur significant and ongoing legal, accounting, and other expenses that we did not incur as a private company. We are subject to the reporting requirements of the Exchange Act, which 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 Select 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. Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the 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. Under the statute, we 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 $1.235 billion or more; (ii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iii) the date on which we are deemed to be a large accelerated filer under the rules of the SEC. We will become a large accelerated filer as of December 31, 2023, and as such we will lose emerging growth status on December 31, 2023. 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. 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 IT 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 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 U.S. 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, 2022, we had U.S. federal and state net operating loss carryforwards of $203.2 million and $164.1 million, respectively, some of which begin to expire in 2034. As of December 31, 2022 and 2021, we also had U.S. federal research and development tax credit carryforwards of $4.6 million and $2.7 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, 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 continue to maintain a full valuation allowance against all of our deferred tax assets. We may release all or a portion of the valuation allowance in the near-term; however, the release of the valuation allowance, as well as the exact timing and the amount of such release, continue to be subject to, among other things, our level of profitability, revenue growth, clinical program progression and expectations regarding future profitability.

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 pharmaceutical 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 we fail to satisfy the continued listing requirements of the Nasdaq Global Select Market, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq 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 Select 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 may 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

During the three months ended June 30, 2023, the following officers of the Company (as defined in Rule 16a-1(f)) adopted the following trading plans for the sale of our common stock pursuant to the terms of the applicable plan; such plans are intended to satisfy the affirmative defense conditions of Rule 10b5–1(c)(1) of the Exchange Act:

- Joshua Cohen, our Co-Chief Executive Officer and a member of our board of directors, adopted a new Rule 10b5-1 trading plan on May 22, 2023, which is scheduled to expire on November 30, 2023. The aggregate number of shares of our common stock authorized to be sold under this new arrangement is 75,000;
- Justin Klee, our Co-Chief Executive Officer and a member of our board of directors, adopted a new Rule 10b5-1 trading plan on May 22, 2023, which is scheduled to expire on November 30, 2023. The aggregate number of shares of our common stock authorized to be sold under this new arrangement is 75,000;
- James Frates, our Chief Financial Officer, adopted a new Rule 10b5-1 trading plan on May 22, 2023, which is scheduled to expire on December 14, 2023. The aggregate number of shares of our common stock authorized to be sold under this new arrangement is 60,000; and
- Gina M. Mazzariello, our Chief Legal Officer and General Counsel, adopted a new Rule 10b5-1 trading plan on May 16, 2023, which is scheduled to expire on March 8, 2024. The aggregate number of shares of our common stock authorized to be sold under this new arrangement is 111,119, which includes shares that may be withheld or sold to cover withholding taxes at the time of vesting.

No other director or officer has adopted or terminated any non-Rule 10b5-1 trading arrangements during the quarter ended June 30, 2023.

Item 6. Exhibits.

Exhibit Number	Description
3.1	Fourth Amended and Restated Certificate of Incorporation of Amylyx Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on January 11, 2022).
3.2	Second Amended and Restated Bylaws of Amylyx Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on January 11, 2022).
10.1*	Commercial Supply Agreement, dated as of August 8, 2023, by and between the Registrant and ICE S.p.A. (formerly Prodotti Chimici e Alimentari S.p.A.).
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.3*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1+	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2+	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.3+	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith.
+ This certification will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, except to the extent specifically incorporated by reference into such filing.
# Indicates a management contract or any compensatory plan, contract or arrangement.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

AMYLYX PHARMACEUTICALS, INC.

Date: August 10, 2023

By: /s/ Joshua B. Cohen
Joshua B. Cohen
Co-Chief Executive Officer

By: /s/ James M. Frates
James M. Frates
Chief Financial Officer

Exhibit 10.1

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 "[***]".

To:

AMYLYX PHARMACEUTICALS, INC.
43 Thorndike Street
Cambridge, MA 02141

Attention:

Joshua Cohen (Co-CEO)

8 August 2023

Dear Sirs,

Commercial Supply Agreement

We acknowledge receipt of your letter including the proposal that Amylyx Pharmaceuticals, Inc. will execute the Commercial Supply Agreement contained thereto and we hereby return to you a copy of such Commercial Supply Agreement duly signed by our authorised signatory in sign of our full, irrevocable and unconditional acceptance thereof.

* * *

"To:

ICE S.p.A.
Via Sicilia 8/10
42122 – Reggio Emilia (RE)

Attention:

Agostino Barazza (CEO)

Dear Sirs,

Commercial Supply Agreement

8 August 2023

Following our recent understandings, please find below the Commercial Supply Agreement that we propose ICE S.p.A.

executes.

*** *** ***

COMMERCIAL SUPPLY AGREEMENT

between

ICE S.p.A.

and

AMYLYX PHARMACEUTICALS, INC.

This supply agreement (the "**Agreement**") is entered into between

(1) ICE S.p.A., a company incorporated under the laws of Italy, registered with the companies register of Reggio Emilia under number 01227810353 and having its registered office in Via Sicilia 8/10, 42122, Reggio Emilia, Italy ("**ICE**"); and

(2) Amylyx Pharmaceuticals, Inc. a company incorporated under the state laws of Delaware, USA, whose registered office is at 43 Thorndike Street, Cambridge, MA 02141 (the "**Customer**" or "**AMYLYX**" and, jointly with ICE, the "**parties**" and each a "**party**").

RECITALS

(A) ICE is a chemical company that, inter alia, carries on the business of manufacturing and supplying the API (as defined below).

(B) The Customer is a pharmaceutical company involved in the development, manufacturing and marketing of medicinal neurodegenerative products.

(C) ICE developed tauroursodeoxycholic acid [***] (as defined below, the API) specifically for the Customer, carrying out significant investments as well as chemical and technical searches, bearing the related costs, for the development of the API.

(D) On December 9, 2019, the parties executed a Research, Development and Supply Agreement (as subsequently amended, the "**RDS Agreement**"), which provided the details of the ICE and Customer relationship in advance of the Finished Product becoming commercialized. At the time, the clinical phases to obtain the relevant regulatory approvals for the pharmaceutical use of the Finished Product (whose costs were borne by ICE) were still ongoing.

(E) Since then:

(a) Amylyx' Finished Product named AMX0035 completed the clinical phases and obtained regulatory approval in the US, thus overcoming the previously existing uncertainty over the timeline of its commercial launch;

(b) The said Finished Product obtained patent protection under Customer's IPR at least until 2040; and

(c) ICE made *inter alia* significant investments in expanding its production capacity as to support the Customer's potential demand.

(F) ICE may also supply the API and/or the Alternative API to the Customer for the development of other innovative medicinal neurodegenerative products to which patent protection may be sought. ICE is willing to assist the Customer in the development of such products, including any relevant regulatory approvals.

(G) The Customer wishes to buy from ICE, and ICE wishes to supply to the Customer, the API on the terms and conditions set out in this Agreement, that shall replace and supersede the RDS Agreement.

Agreed terms

1. Interpretation

The following definitions and rules of interpretation in this clause apply in this Agreement.

1.1 Definitions. In addition to the terms elsewhere defined in this Agreement, the following words and expressions shall be given the meaning ascribed to each of them below:

Affiliate: with respect to an entity, any other entity that directly or indirectly (through, inter alia, one or more entities) Controls or is Controlled by, or is under common Control with, that first entity. **Control** shall mean (i) the holding of the majority of the voting rights, or (ii) the right to appoint or remove a majority of board of directors or equivalent managing body. **Controlling** and **Controlled** shall be construed accordingly.

Alternative API: an active pharmaceutical ingredient chemically identical to the API, but manufactured by an alternative route or using alternative raw materials than the API.

API: the active pharmaceutical ingredient, which for the purpose of this Agreement shall mean tauroursodeoxycholic acid [***].

Apparent Defect: any Defect that can be detected by visual inspection of the API and/or any release analysis

of the API to be carried out by the Customer.

Applicable Laws: the laws, regulations and mandatory rules and guidance in force in the territory in which the Customer intends to distribute and sell the Finished Product, as amended and updated from time to time.

Business Day: a day, other than a Saturday, Sunday or public holiday in Italy and/or the United States.

Calendar Day: means the twenty-four (24) hour period from midnight to midnight and includes Saturday, Sundays an all public holidays.

Calendar Year: means the one-year (1) year period from January 1 to December 31.

Commencement Date: August 8, 2023.

Defect: a material non-conformity of the API to the Specification on Delivery and "**Defective**" shall be construed accordingly.

Delivery: completion of delivery of API specified in an Order in accordance with clause 7.2 or clause 7.4(a) and "**Delivered**" shall be construed accordingly.

Delivery Date: the date on which the API specified in the Order will be ready for collection as confirmed by ICE in accordance with clause 9.2(b).

Delivery Location: ICE Facility, at via Novi 78, 15060 Basaluzzo, AL, Italy.

Facility: the facility owned and/or operated by ICE at which the API is manufactured.

Finished Product: any Customer's products for the treatment of neurological diseases or disorders that contains the API or Alternative API manufactured under this Agreement.

Force Majeure Event: has the meaning given in clause 23.1.

Foreground IPRs: all new Intellectual Property Rights that are generated by or on behalf of ICE in the course of the performance of its obligations under this Agreement, including any improvement, development, enhancement, modification or derivative of the Background IPRs, of the API or of any proprietary aspect of ICE's manufacturing process for the API.

Group: in relation to a company, that company and any Affiliate, from time to time, of that company.

ICE Background IPRs: all Intellectual Property Rights of which ICE, or a member of ICE's Group, is the owner or licensee, and which existed before the Commencement Date or were generated independently of this Agreement, and are used by ICE in the performance of its obligations under this Agreement, including all Intellectual Property Rights in or arising out of the API and ICE's manufacturing process for the API.

Initial Forecast: the agreed forecast of the Customer's API demand from the Commencement Date until [***], a copy of which is attached as Schedule 2.

Intellectual Property Rights or IPRs: patents, utility models, rights to inventions, copyright and neighbouring and related rights, moral rights, trade marks and service marks, business names and domain names, rights in get-up and trade dress, goodwill and the right to sue for passing off or unfair competition, rights in designs, rights in computer software, database rights, rights to use, and protect the confidentiality of, confidential information (including know-how and trade secrets), and all other intellectual property rights, in each case whether registered or unregistered and including all applications and rights to apply for and be granted, renewals or extensions of, and rights to claim priority from, any rights and all similar or equivalent rights or forms of protection that subsist or will subsist now or in the future in any part of the world.

Joint Steering Committee (JSC): has the meaning as defined in clause 22.1 of this Agreement.

Latent Defect: any Defect that cannot be detected by visual inspection of the API and/or any release analysis to be carried out by the Customer.

Mutual Confidentiality Agreement or CDA: the non-disclosure agreement signed by the parties on [***] and attached hereto as Schedule 3.

Order: an order for API submitted by the Customer in accordance with clause 9.

Price (or Prices): the price or prices of the API as determined in accordance with clause 5.1.

Quality Agreement: the agreement entered into on the date hereof that, inter alia, which includes the Specifications of the API and defines the responsibilities relative to quality tasks to assure the manufacture, supply and use of safe materials acceptable for pharmaceutical use.

Regulatory Authority: any competent regulatory authority with jurisdiction over the manufacturing by ICE of the API at the Facility or the supply by the Customer of the Finished Products in any of the main markets, which include, but not limited to the European Medicines Agency, Health Canada, the Pharmaceuticals and Medical Devices Agency of Japan and the United States Food and Drug Administration, and in each case including any successor regulator from time to time.

Specifications: the specifications of the API set forth within, and modified from time to time, in the Quality Agreement.

Term: the Initial Term as may be renewed.

1.2 Clause, Schedule and paragraph headings shall not affect the interpretation of this Agreement.

1.3 A **person** includes a natural person, corporate or unincorporated body (whether or not having separate legal personality).

1.4 The Schedules form part of this Agreement and shall have effect as if set out in full in the body of this Agreement and any reference to this Agreement includes the Schedules.

1.5 A reference to a **month** shall be a reference to a calendar month and a reference to a **year** shall be a reference to a calendar year.

1.6 Unless the context otherwise requires, words in the singular shall include the plural and vice versa.

1.7 This Agreement shall be binding on, and inure to the benefit of, the parties to this Agreement and their respective personal representatives, successors and permitted assigns, and references to any party shall include that party's personal representatives, successors and permitted assigns.

1.8 A reference to a statute or provision is a reference to it as amended, extended or re-enacted from time to time.

1.9 A reference to a statute or provision shall include all subordinate legislation made from time to time under that statute or provision.

1.10 References to clauses and Schedules are to the clauses and Schedules of this Agreement; and references to paragraphs are to paragraphs of the relevant Schedule.

1.11 Any words following the terms **including**, **include**, **in particular**, **for example** or any similar expression shall be construed as illustrative and shall not limit the sense of the words, description, definition, phrase or term preceding those terms.

2. Subject matter

This Agreement sets out the terms and the conditions that apply to the sale by ICE to the Customer, and the purchase by the Customer from ICE, of the API during the Term.

SECTION I: COMMERCIAL TERMS

3. Commencement and Term

This Agreement shall begin on the Commencement Date and shall continue until 31 December 2028 (the "**Initial Term**"). At the conclusion of the Initial Term the, the JSC shall determine whether this Agreement shall be renewed, according to the provisions of clause 22 below.

4. Supply of products

4.1 During the Term:

(a) the Customer shall purchase all of its requirements for the API on an exclusive basis from ICE;

(b) ICE shall sell the API to the Customer on an exclusive basis only for the purpose of allowing the Customer to conduct their development and commercial programs to treat any neurological disease or disorder in accordance with clause 4.3.

It is agreed between the Parties that, should the Customer use an Alternative API to produce AMX0035 and/or other finished products to treat any neurological disease or disorder, the Customer shall purchase all of its requirements for the Alternative API on an exclusive basis from ICE and ICE shall sell the Alternative API to the Customer on an exclusive basis only for the purpose of allowing the Customer to conduct their development and commercial programs to treat any neurological disease or disorder in accordance with clause 4.3. In such a case, the terms and conditions of this Agreement shall also apply to the Alternative API and any reference to the API shall be deemed made (also) to the Alternative API.

It is further understood that ICE shall be free to sell the API (and, in case, the Alterative API) to any third party that, after due enquiry, declares that it does not intend to use the API for the treatment of neurological diseases or disorders.

4.2 During the Term, ICE shall supply Customer such quantities of API as the Customer may order, provided that:

(a) except for the Calendar Year 2023 (for which the provisions of letter (b) below apply), the minimum amount of the API that the Customer shall purchase in each calendar year from ICE is equal to [***];

(b) exclusively with reference to the period from the Commencement Date until 31 December 2023, AMYLYX shall purchase from ICE [***] of API; and

(c) in case the Customer needs in any Calendar Year (different from 2023) more than [***] of API, it shall let ICE know with a written notice with [***] written notice. In this case, ICE shall be allowed to sell to the Customer the API produced in other facilities of the group to which ICE belongs and the parties shall cooperate to this end.

4.3 Customer shall use the API for the sole purpose of manufacturing and commercializing the Finished Product to treat neurological diseases or disorders.

5. Prices

5.1 The Price for the entire Term shall be the price set out in Schedule 1.

5.2 The Price is exclusive of any amounts in respect of any and all taxes and tariffs. The Customer shall, on receipt of an invoice for the same from ICE, pay to ICE any additional amounts in respect of any and all taxes and tariffs as are chargeable on a supply of the API.

6. Terms of payment

6.1 ICE shall be entitled to invoice the Customer for each Order on or at any time after Delivery.

6.2 The Customer shall pay all invoices (including disputed invoices) in full and in cleared funds within [***] days of the date of such invoices, without any set-off, counterclaim, deduction or withholding. Payment shall be made to the bank account nominated in writing by ICE. All payments under this Agreement shall be in United States Dollars (USD). In case of delayed payments, on any due amount interests in an amount equal to [***] per month shall automatically apply.

6.3 All payments payable to ICE by the Customer under this Agreement shall become immediately due and payable on expiry or termination of this Agreement for any reason. This clause 6.3 is without prejudice to any right to claim for interest under the law or under this Agreement.

6.4 The Customer cannot raise objections and exceptions (including breaches of this Agreement by ICE and/or the existence of any dispute on any invoice) to avoid or delay the payment of any invoice, provided that the Client shall have the right to dispute an invoice. In such a case, Customer will notify ICE within [***] days of receipt of the invoice containing the disputed amount, and the parties will then work together in good faith, to resolve the dispute.

7. Delivery

7.1 The Customer will collect released API, in accordance with EXW – INCOTERMS 2020, from the Delivery Location on the Delivery Date.

7.2 Delivery is completed when ICE places the API at the Customer's disposal at the Delivery Location. At the scheduled time for Delivery, the Customer or its carrier may enter the Delivery Location provided that the attendees comply with site rules provided by or on behalf of ICE and any specific directions given to the attendees while on-site at the Delivery Location. Collection activities shall be made in the shortest time possible by qualified personnel.

7.3 ICE shall have no liability for any failure or delay in delivering an Order to the extent that any failure or delay is caused by the Customer's negligence or failure to comply with its obligations under this Agreement or by a Force Majeure Event.

7.4 If the Customer fails to take delivery of an Order in accordance with clause 7.1, then, except where that failure or delay is caused by ICE's failure to comply with its obligations under this Agreement:

(a) delivery of the Order shall be deemed to have been completed at 6.00pm CET on the Delivery Date; and

(b) ICE shall store the Order until delivery takes place and charge the Customer for all related costs and expenses (including insurance).

8. Forecasts

8.1 Without prejudice to the Customer's obligations under clause 4.1(a), the Parties agree that:

(a) the Initial Forecast provides the forecast of the Customer's API demand until [***];

(b) the [***] months of the Initial Forecast (the "**First Period Committed Amounts**") are binding upon the Customer and ICE;

(c) Within [***] days from the Commencement Date, and then subsequently within [***] days of the commencement of each month for the entire Term, transmit a forecast of its requirements of API and of estimated delivery dates for the following [***] month period (each, a "**Rolling Updated Forecast**") in writing, which covers the [***] period starting from the month immediately following the one when the Rolling Updated Forecast is issued and which:

(i) shall not amend the quantities of the residual [***] binding portion of any previous forecast;

(ii) add [***] whose quantities are binding upon the Customer only (the "**Rolling Binding Month**"); and

(iii) provide for an updated non-binding forecast of the subsequent [***] period;

(d) ICE shall be free to accept the quantities of the Rolling Binding Month included in any Rolling Updated Forecast, provided that such acceptance shall not be unreasonably withheld by ICE. ICE shall notify the Customer within [***] Business Days of receiving each Rolling Updated Forecast whether it accepts the quantities included in the Rolling Binding Month. In lack of any reply, or if ICE does not accept the Rolling Binding Month, the parties shall negotiate in good faith to attempt to agree the Rolling Binding Month promptly;

(e) upon acceptance by ICE of the quantities included in any Rolling Binding Month, such Rolling Binding Month shall become binding upon the Parties; and

(f) the Customer shall act in good faith when forecasting its requirements for API.

8.2 It is agreed that:

(a) the Customer shall issue to ICE Orders that confirm:

(i) the quantities of API included in the First Period Committed Amount, within [***] Calendar Days of the Commencement Date ; and

(ii) any agreed Rolling Binding Month, within [***] Calendar Days of the date when ICE accepts the Rolling Binding Month;

(b) ICE shall confirm in writing the acceptance of said Orders within [***] Business Days of receipt (such confirmation shall not be withheld to the extent the Orders effectively reflect the First Period Committed Amounts and each agreed Rolling Binding Month) by issuing an order number. No such Order shall be deemed to be accepted by ICE until it formally replies to the Customer in this respect; and

(c) the Customer shall ensure that all such Orders are submitted in all other respects in accordance with clause 9.

9. Orders

9.1 Without prejudice to the provisions of clause 8, each Order, shall also:

(a) be given in writing;

(b) specify the type and quantity of the API ordered; and

(c) specify the Customer's proposed Delivery Date on which the API specified in the Order is to be ready for collection, which shall be at least [***] days after the date of deemed receipt of the Order by ICE.

9.2 Without prejudice to the provisions of clause 8, ICE shall notify the Customer in writing whether it accepts the Order and such notification shall include:

(a) the Order number; and

(b) the confirmed Delivery Date on which the API specified in the Order will be ready for collection.

9.3 In no event shall any terms or conditions included by the Customer on any Order purchase order, invoice or acknowledgement thereof or any other document, whether paper, electronic or otherwise, relating thereto, apply to the relationship between the parties under this Agreement, unless such terms are expressly agreed to by the parties in writing.

9.4 If there is any conflict between the terms of any Order and the terms of this Agreement, the terms of this Agreement shall prevail.

10. Title and risk

10.1 Risk in the API shall pass to the Customer on Delivery, except where it passes earlier under clause 7.4 due to non-collection.

10.2 Title to the API delivered to the Customer shall pass to the Customer on Delivery.

SECTION II: QUALITY ASPECTS

11. Acceptance and defective products

11.1 ICE represents, warrants that the API supplied to the Client will be free from Defects.

11.2 If a Delivery of API is Defective, then the Client shall have the right, as exclusive remedy, to reject such non-conforming Delivery of API.

11.3 The Customer may reject any Defective API Delivered to it, provided that:

(a) written notice of rejection is given to ICE in accordance with clause 11.4; and

(b) none of the events listed in clause 11.5 apply.

11.4 If the Customer wishes to reject any delivery of the API:

(a) on the basis of an Apparent Defect, it shall notify ICE within [***] Business Days after Delivery of the API;

(b) on the basis of a Latent Defect, it shall notify ICE within [***] Business Days after the Customer's discovery of the same.

Any such notification must be in writing and include a detailed indication of the reasons for rejection (including details and evidence of any tests or inspections carried out, the associated results and observations, and any other basis for concluding that the API is Defective) and evidence that the Customer has complied with all the necessary and appropriate measures and procedure for the storage and preservation of the API in order to preserve its integrity and characteristics. Unless the Customer provides the above-mentioned written notification within the applicable above-mentioned timescale, the Customer shall be deemed to have accepted the API and shall not be entitled to reject the same.

11.5 The events referred to in clause 11.3(b) comprise the following:

(a) the Customer has not complied with all the necessary and appropriate measures and procedure for the storage and preservation of the API in order to preserve its integrity and characteristics;

(b) any Latent Defect was not discovered within [***] months of Delivery of the API;

(c) the Customer makes any further use of the API after giving notification in accordance with clause 11.4;

(d) the relevant Defect was caused by any act, omission or circumstance that takes place after the API is delivered;

(e) the Customer or its agent or contractor alters, repairs, reprocesses or reworks that API without the written consent of ICE; or

(f) the API differs from the Specification because of changes made to ensure compliance with Applicable Laws or regulatory requirements.

11.6 Within [***] Business Days after any notification in writing from the Customer referred to under clause 11.4, and provided that the conditions set out under clause 11.5 are met, the Customer shall deliver to ICE a sample of the allegedly Defective API (the "**Sample**").

11.7 Within [***] Business Days after receipt of the Sample by ICE (the "**Evaluation Period**"), ICE will perform the relevant controls on its retained samples of the API and on the Sample to evaluate the claimed Defect and determine whether such API is Defective. If ICE, after performing the relevant controls, confirms that the API is Defective, ICE shall, as it determines:

(a) within [***] Business Days after the end of the Evaluation Period, replace the Defective API with conforming API; or

(b) deduct the price paid by Customer for the Defective API from its next invoice to the Customer or send the Customer a credit note for the same amount.

Lack of any reply by ICE within the Evaluation Period implies that, according to ICE, the API at hand is not Defective.

11.8 Should any dispute arise between the parties in relation to the existence of the claimed Defects in the API, such dispute shall be discussed by the JSC, that shall be convened for this purpose within [***] Business Days of the expiry of the Evaluation Period. The JSC shall try to find a mutually satisfactory solution to the matter within [***] Business Days of the expiry of the Evaluation Period. In case the JSC is not able to find a mutually satisfactory solution, the issue shall be resolved by a third-party expert or an independent testing entity of international recognised reputation within the pharmaceutical industry (the "**Expert**"), selected by the JSC

and jointly appointed by the Parties. Each party agrees to cooperate with the Expert and provide to the Expert, samples of any API in its possession that are the subject of the dispute to allow any testing required by the Expert. Each party agrees to act promptly to meet its obligations under this clause and bear the cost of doing so. The Expert shall decide the dispute on the basis of the Specification and his decision, which will be notified to the Parties in writing, shall be final and binding on the Parties and may not be challenged by the same (except in the case of manifest error). The parties will not progress any substantive proceedings in the courts until the applicable procedure under this clause is completed, except to the extent that a party's right to issue proceedings would be prejudiced by delay. The expenses of the Expert shall be borne by the party against whom the Expert's decision is rendered. In the event that the Expert determines that the API is Defective, ICE shall, as it determines:

(a) within [***] Business Days after the Expert's decision is notified to the parties, replace the Defective API with conforming API; or

(b) deduct the price paid by Customer for the Defective API from its next invoice to the Customer or send the Customer a credit note for the same amount.

11.9 Where ICE confirms that the API is Defective under clause 11.7, or the Expert determines the same under clause 11.8, once ICE has complied with its obligation to replace the Defective API, deduct the price paid for the Defective API from its next invoice or send the Customer a credit note (at ICE's determination), it will have no further liability to the Customer (whether based on contract, tort, negligence, or otherwise) for the Defective API and any failure that gave rise to the relevant right of rejection. The terms of this Agreement will apply to any replacement API supplied by ICE.

11.10 To the maximum extent permitted by the law, the remedies provided in this clause 11 shall be exclusive and in lieu of any other right, action, defence, claim or remedy of the Customer provided by the law or otherwise (including this Agreement) in connection with or by virtue of the presence of Defects in the API. In particular, for the avoidance of doubts, to the maximum extent permitted by the law, the parties agree that: (a) no right, action, defence, claim or remedy shall be available to the Customer in case of defects of the API different from the Defects and (b) ICE does not represent and warrant, nor undertake, that the API is fit for the manufacturing and sale of any product by the Customer and/or for any other use the Customer intends to make of the API.

11.11 For the avoidance of doubts, the fact that the Customer has made a claim for Defects pursuant to this clause 11 shall not relive the same from its payment obligations hereunder.

SECTION III: INDEMNIFICATION PROVISIONS

12. Indemnity

12.1 Each party (the "**Indemnifying Party**") shall indemnify and hold the other party and its officers, directors and employees (the "**Indemnified Party**") harmless from and against any and all third party damage, liability and cost, including court costs and all reasonable legal fees (collectively "**Claims**"), which in each case result from or arises directly out of the Indemnifying

Party's material breach of this Agreement to the extent caused by gross negligence, fraud or willful misconduct of the Indemnifying Party.

12.2 The Indemnifying Party's liability under the indemnity in clause 12.1 is conditional on the Indemnified Party discharging the following obligations. If any third party makes a Claim that may reasonably be considered likely

to give rise to a liability under the indemnity in clause 12.1, the Indemnified Party shall:

(a) as soon as reasonably practicable, give written notice of the Claim to the Indemnifying Party, specifying the nature of the Claim in reasonable detail;

(b) not settle the Claim nor make any admission of liability, agreement or compromise in relation to the Claim without the prior written consent of the Indemnifying Party;

(c) at the Indemnifying Party's expense, cooperate fully with the Indemnifying Party and its professional advisers in relation to the conduct of the Claim, including by giving the Indemnifying Party and its professional advisers access at reasonable times (on reasonable prior notice) to its premises and its officers, directors, employees, agents, representatives or advisers, and to any relevant assets, accounts, documents and records within the power or control of the Indemnified Party, to enable the Indemnifying Party and its professional advisers to examine them and to take copies (at the Indemnifying Party's expense) in order to assess the Claim; and

(d) be deemed to have given the Indemnifying Party sole control and authority to avoid, dispute, settle, compromise or defend the Claim, provided that the Indemnifying Party:

(i) shall keep the Indemnified Party duly informed on the state of the Claim; and

(ii) shall not agree, without the prior written consent of the Indemnified Party (not to be unreasonably withheld or delayed), to any settlement of the Claim that does not include a complete release of the Indemnified Party from all liability with respect thereto.

In case it is not possible to apply the provisions of this clause 12.2 for reasons connected to the procedural rules applicable to the claim, the Parties shall act so that the substantial content of this clause is preserved.

12.3 The Indemnified Party shall, in relation to any loss or damage it may suffer or incur as a result of an event that may give rise to a claim under the indemnity in clause 12.1, take all reasonable steps to avoid or mitigate that loss or damage including by pursuing any relevant third party, or claiming under any relevant insurance policy for the loss or damage.

13. Limitation of liability

13.1 SUBJECT ALWAYS TO CLAUSE 13.5, THE AGGREGATE AMOUNT OF ALL LIABILITIES OF A PARTY ARISING UNDER OR IN CONNECTION WITH THIS AGREEMENT AND/OR THE QUALITY AGREEMENT, INCLUDING THE INDEMNIFICATION OBLIGATIONS UNDER CLAUSE 12.1 OF THIS AGREEMENT, IN EACH CASE (WHETHER ARISING FROM BREACH OF CONTRACT (INCLUDING NEGLIGENT BREACH OF CONTRACT), TORT (INCLUDING NEGLIGENCE), BREACH OF STATUTORY DUTY, MISREPRESENTATION, RESTITUTION OR OTHERWISE) SHALL BE LIMITED,

IN ANY GIVEN CALENDAR YEAR, TO [***], PROVIDED THAT, IN ANY EVENT, SUCH LIABILITY SHALL NOT EXCEED IN THE AGGREGATE [***].

13.2 SUBJECT ALWAYS TO CLAUSE 13.5, IN NO EVENT (WHETHER ARISING FROM BREACH OF CONTRACT (INCLUDING NEGLIGENT BREACH OF CONTRACT), TORT (INCLUDING NEGLIGENCE), BREACH OF STATUTORY DUTY, MISREPRESENTATION, RESTITUTION OR OTHERWISE) SHALL EITHER PARTY BE LIABLE UNDER OR IN CONNECTION WITH THIS AGREEMENT AND/OR THE QUALITY AGREEMENT FOR ANY:

(A) LOSS OF PROFIT;

(B) LOSS OF USE OF OR CORRUPTION OF SOFTWARE, DATA OR INFORMATION;

(C) LOSS OF USE, LOSS OF SALES, LOSS OF REVENUE, LOSS OF PRODUCTION OR LOSS OF BUSINESS;

(D) LOSS OF AGREEMENTS OR CONTRACTS;

(E) LOSS OF ANTICIPATED SAVINGS;

(F) LOSS OF OR DAMAGE TO GOODWILL OR REPUTATION; OR

(G) INDIRECT OR CONSEQUENTIAL LOSS.

13.3 NOTHING IN THIS CLAUSE 13 SHALL LIMIT THE CUSTOMER'S OBLIGATION OR LIABILITY TO PAY FOR API SUPPLIED TO IT IN ACCORDANCE WITH THIS AGREEMENT.

13.4 WITHOUT PREJUDICE TO THE ABOVE EXCLUSIONS AND RESTRICTIONS, BUT SUBJECT ALWAYS TO CLAUSE 13.5, A PARTY SHALL NOT BE LIABLE UNDER OR IN CONNECTION WITH THIS AGREEMENT (INCLUDING WITH RESPECT TO THE INDEMNIFICATION OBLIGATIONS UNDER CLAUSE 12.1) AND/OR THE QUALITY AGREEMENT (WHETHER ARISING FROM BREACH OF CONTRACT (INCLUDING NEGLIGENT BREACH OF CONTRACT), TORT (INCLUDING NEGLIGENCE), BREACH OF STATUTORY DUTY, MISREPRESENTATION, RESTITUTION OR OTHERWISE) FOR ANY CLAIM THAT IS NOTIFIED TO THAT PARTY LATER THAN 1 YEAR AFTER THE EXPIRY OF THIS AGREEMENT.

13.5 NOTHING IN THIS AGREEMENT WILL OPERATE TO EXCLUDE OR RESTRICT ANY LIABILITY OF A PARTY FOR FRAUD OR FRADULENT MISREPRESENTATION, DEATH OR PERSONAL INJURY CAUSED BY A PARTY'S NEGLIGENCE OR ANY MATTER FOR WHICH IT IS NOT PERMITTED BY LAW TO EXCLUDE OR LIMIT, OR TO ATTEMPT TO EXCLUDE OR LIMIT, ITS LIABILITY.

14. Insurance

14.1 During the Term, each party shall maintain in force appropriate insurance cover in respect of its potential liabilities arising out of this Agreement. In particular, each party shall keep a third party damage insurance equal to [***]. Each party shall provide to the other, promptly on written request, copies of the insurance policy certificates in respect of such cover and reasonable evidence that the policies to which those certificates relate remain in force.

SECTION IV: OTHER PROVISIONS

15. Assistance with registration

15.1 ICE undertakes to use its reasonable commercial efforts to support the Customer in the registration process of the Finished Product before any Regulatory Authority. In particular, ICE undertakes, at the Customer's request, to submit the API Drug Master File before any Regulatory Authority in the US, EU, Canada and Japan, at ICE's costs. ICE's support with respect to the registration submission in any other country as well as any other supportive activity done by ICE shall be discussed in good faith between the parties, provided that in no event shall ICE bear the cost of any such activity.

16. Assignment and other dealings

16.1 Subject to clause 16.2, neither party shall novate, assign, transfer, mortgage, charge, subcontract, delegate, declare a trust over or deal in any other manner with any or all of its rights and obligations under this Agreement without the prior written consent of the other party (such consent not to be unreasonably withheld or delayed).

16.2 Notwithstanding the foregoing, each party may, without consulting or obtaining consent from the other, at any time assign or otherwise transfer this Agreement and/or any of its rights and obligations hereunder to any person to which it transfers all, or substantially all, of its business or that part of its business to which this Agreement relates (the "**Transferee**") provided that the Transferee undertakes in writing to the other party to be bound by the transferor's obligations under this Agreement. The transfer can be prevented only if the non-transferring party can demonstrate that there are objectively justifiable reasons why further to the transfer of the Agreement the Transferee would not be able to comply with the terms of the Agreement.

17. Confidentiality

17.1 The terms of the Mutual Confidentiality Agreement shall govern the confidentiality and non- disclosure obligations of the parties and shall be deemed as an integral part of this Agreement and attached hereto as Schedule 3. For clarity, the parties agree that the CDA shall apply to this Agreement for the entire Term.

17.2 The parties hereby agree that regardless of the content of the Mutual Confidentiality Agreement, each party shall be allowed to disclose this Agreement (except for the Initial Forecast and any updates thereof, and any other forecasts) to any third party interested in directly or indirectly investing in or acquiring such party and/or its business to which this Agreement relates, provided that any such third party shall be bound by confidentiality obligations substantially analogous to those set out under the Mutual Confidentiality Agreement. In such a case, the disclosing party shall inform the other party in advance of such disclosure.

18. Intellectual Property Rights

18.1 The Customer acknowledges that the ICE Background IPRs are and remain the exclusive property of ICE or, where applicable, the third party licensor from whom ICE derives the right to use them.

18.2 The parties acknowledge and agree that, as between ICE and the Customer, the IPRs relating to the Finished Product (excluding the ICE Background IPRs) exclusively belong to Customer.

18.3 Without prejudice to clause 18.2, each party undertakes not to use the other party's name, trademark or corporate name and not to refer to 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.

18.4 In any case, neither party shall make any use of the other party's name, trademark or corporate name in a way that might result, either directly or indirectly, in harm to the other party's business or products.

19. Termination and Suspension

19.1 Without affecting any other right or remedy available to it, either party may terminate this Agreement with immediate effect by giving written notice to the other party if:

(a) the other party fails to pay any undisputed amount due under this Agreement on the due date for payment and remains in default not less than [***] days after being notified in writing to make such payment;

(b) the other party commits a material breach of any other term of this Agreement and that breach is irremediable or (if that breach is remediable) fails to remedy that breach within a period of [***] days after being notified in writing to do so. For the purposes of this clause 19, a breach shall be considered

capable of remedy if the party in breach can comply with the relevant provision in all respects other than as to time of performance;

(c) the other party files for bankruptcy, becomes insolvent or ceases to carry on all or substantially the whole of its business; or

(d) any Force Majeure Event prevents, hinders or delays the other party's performance of its obligations under this Agreement for a continuous period of more than four (4) months.

19.2 Without limiting its other rights or remedies, ICE may suspend provision of the API under this Agreement subject to any of the events listed below:

(a) the Customer suspends, or threatens to suspend, payment of its debts or is unable to pay its debts as they fall due or admits inability to pay its debts or is deemed unable to pay its debts under relevant laws;

(b) the Customer begins negotiations with all or any class of its creditors with a view to rescheduling any of its debts, or makes a proposal for or enters into any compromise or arrangement with any of its creditors other than for the sole purpose of a scheme

for a solvent amalgamation with one or more other companies or the solvent reconstruction;

(c) the Customer applies to court for, or obtains, a moratorium under relevant laws;

(d) a petition is filed, a notice is given, a resolution is passed, or an order is made, for or in connection with the winding up of Customer other than for the sole purpose of a scheme for a solvent amalgamation of the Customer with one or more other companies or the solvent reconstruction of the Customer;

(e) an application is made to court, or an order is made, for the appointment of an administrator or a notice of intention to appoint an administrator is given or an administrator is appointed over the Customer;

(f) the holder of a qualifying floating charge over the assets of the Customer has become entitled to appoint or has appointed an administrative receiver;

(g) a person becomes entitled to appoint a receiver over all or any of the assets of the Customer or a receiver is appointed over all or any of the assets of the Customer;

(h) a creditor or encumbrancer of the Customer attaches or takes possession of, or a distress, execution, sequestration or other such process is levied or enforced on or sued against, the whole or any part of its assets and such attachment or process is not discharged within [***];

occurs, or proceeding is taken, with respect to the other party in any jurisdiction to which it is subject that has an effect equivalent or similar to any of the events mentioned in this clause, or ICE reasonably believes that the Customer is about to become subject to any of them, or if the Customer fails to pay any amount due under this Agreement on the due date for payment.

19.3 ICE may terminate this Agreement with immediate effects in case of breaches by the Customer of clauses 4.1 and 30.1, without prejudice to any damage compensation.

20. Obligations on termination

On termination or expiry of this Agreement, the Customer shall pay any sums due under this Agreement and

each party, upon request of the other, shall promptly return to the other party all equipment, materials and property belonging to the other party that the other party had supplied to it or a member of its Group in connection with the supply and purchase of the API under this Agreement.

21. Survival

21.1 Termination or expiry of this Agreement shall not affect the operation of the following clauses:

(a) clause 12 (Indemnity);

(b) clause 13 (Limitation of liability);

(c) clause 17 (Confidentiality);

(d) clause 20 (Obligations on termination);

(e) clause 34 (Governing law); and

(f) clause 35 (Dispute resolution and arbitration),

and any other provision of this Agreement that is intended to continue to have effect after it has come to an end.

21.2 Termination or expiry of this Agreement shall not affect any rights, remedies, obligations or liabilities of the parties that have accrued up to the date of termination or expiry to the extent necessary to the intended preservation of such rights, remedies, obligations or liabilities, including the right to claim damages for any breach of this Agreement that existed at or before the date of termination or expiry.

22. The Joint Steering Committee (JSC)

22.1 Within [***] of the Commencement Date, the parties shall, according to the rules set out below, establish a Joint Steering Committee ("**JSC**") that shall meet and resolve upon the matters described in this clause 22.

22.2 Membership

(a) Members. ICE and Customer shall each appoint three (3) representatives to serve on the JSC and all said representatives shall have voting rights to the JSC ("**JSC Member**"). ICE and Customer shall also each appoint one JSC Member to serve as a Co-Chairperson and serve until removed by the appointing party. Each party can at any time replace any of the JSC Member appointed by the same party by giving written notice to the other party. Each party can also appoint for each meeting an observer that can attend the relevant meeting.

(b) Scope. The JSC Members shall meet to discuss and decide upon the following matters:

- the capacity plan, regulatory strategy, and future development(s) under this Agreement;
- any renewal and/or extension of the term of this Agreement;
- global regulatory submissions to support Amylyx' expansion plans for its finished products;
- any disagreement upon the existence of Defects in the API according to clause 11.

22.3 Meetings and Voting

(a) Cadence and Scheduling. Meetings shall be held when mutually decided by the Co- Chairperson, but in no event less than annually. All members shall be invited to each meeting in writing.

(b) Quorum and Voting. The presence of two (2) JSC Members appointed by each of ICE and Customer shall constitute a quorum for the transaction of business at any meeting of the JSC. All decisions of the JSC shall be made by unanimous decision of all the JSC Members (i.e., 6 members out of 6). If a vote is brought to the table at any JSC meeting and all 6 members are not in attendance to vote, an ad-hoc meeting shall be scheduled within thirty (30) days of said meeting. If JSC Members cannot

agree on a matter, said matter shall be escalated to the CEOs of both Customer and ICE. If a disagreement on a matter continues for thirty (30) days, formal proceedings outside the JSC may commence. Any decision taken by the JSC as per above and recorded in duly drafted and executed minutes as per below shall be binding upon the parties.

(c) Recording. Minutes of each meeting of the JSC shall be drafted within 5 Business Days of the date of the meeting and circulated to all JSC Members for their comments. The minutes shall record: a) who is in attendance, b) the items discussed,
c) how vote was expressed and d) the decisions taken. The agreed upon version of the minutes shall be signed by all JSC Members in attendance at the relevant meeting.

23. Force majeure

23.1 **Force Majeure Event** means any circumstance not in a party's reasonable control including:

(a) acts of God, flood, drought, earthquake or other natural disaster;

(b) epidemic or pandemic;

(c) terrorist attack, civil war, civil commotion or riots, war, threat of or preparation for war, armed conflict, imposition of sanctions, embargo, or breaking off of diplomatic relations;

(d) nuclear, chemical or biological contamination, or sonic boom;

(e) any law or any action taken by a government or public authority, including imposing an export or import restriction, quota or prohibition, or failing to grant a necessary licence or consent;

(f) collapse of buildings, fire, explosion or accident;

(g) any labour or trade dispute, strikes, industrial action or lockouts; and

(h) interruption or failure of utility service.

For the avoidance of doubts, it is hereby clarified that the fact that any authority denies the approval to the commercialization of any Finished Product in any geography or the subsequent withdrawal or cancellation of any such approval does not represent a Force Majeure Event.

23.2 Provided it has complied with clause 23.3, if a party is prevented, hindered or delayed in or from performing any of its obligations under this Agreement (other than an obligation to make payment) by a Force Majeure Event ("**Affected Party**"), the Affected Party shall not be in breach of this Agreement or otherwise liable for any such failure or delay in the performance of such obligations. The time for performance of such obligations shall be extended accordingly for a reasonable period of time.

23.3 The Affected Party shall:

(a) as soon as reasonably practicable after the start of the Force Majeure Event, notify the other party of the Force Majeure Event, the date on which it started, its likely or

potential duration, and the effect of the Force Majeure Event on its ability to perform any of its obligations under this Agreement; and

(b) use all reasonable endeavours to mitigate the effect of the Force Majeure Event on the performance of its obligations.

24. General warranties

24.1 Each party warrants and represents that:

(a) it has full capacity and authority to enter into and perform this Agreement;

(b) this Agreement is executed by a duly authorised representative of that party;

(c) once duly executed, this Agreement will constitute its legal, valid and binding obligations;

(d) it is not a party to or otherwise subject to any contract or arrangement with a third party that would prevent, prohibit, frustrate or materially conflict with any of its obligations or the other party's rights under this Agreement; and

(e) financial information relating to that party or its affairs that it disclosed to the other party before execution of the agreement is accurate, not misleading and without material omissions.

25. Costs

Except as expressly provided in this Agreement, each party shall pay its own costs incurred in connection with the negotiation, preparation and execution of this Agreement.

26. Order of preference and severance

26.1 This Agreement shall take precedence over any pre-printed terms and conditions contained in the Parties' purchase orders issued after the Commencement Date, acknowledgements or other forms, although such other documents will remain in force to the extent they do not conflict with the terms and conditions of this Agreement.

26.2 If any provision or part-provision of this Agreement is or becomes invalid, illegal or unenforceable, it shall be deemed deleted, but that shall not affect the validity and enforceability of the rest of this Agreement.

26.3 If any provision or part-provision of this Agreement is deemed deleted under clause 26.2, the parties shall negotiate in good faith to agree a replacement provision that, to the greatest extent possible, achieves the intended commercial result of the original provision.

27. Further assurance

At its own expense, each party shall, and shall use all reasonable endeavours to procure that any necessary third party shall, promptly execute and deliver such documents and perform such acts as may be required for the purpose of giving full effect to this Agreement.

28. Variation

No variation of this Agreement shall be effective unless it is in writing and signed by both parties (or their

authorised representatives).

29. Waiver

29.1 A waiver of any right or remedy under this Agreement or by law shall only be effective if given in writing and shall not be deemed a waiver of any subsequent right or remedy.

29.2 A failure or delay by a party to exercise any right or remedy provided under this Agreement or by law shall not constitute a waiver of that or any other right or remedy, nor shall it prevent or restrict any further exercise of that or any other right or remedy. No single or partial exercise of any right or remedy provided under this Agreement or by law shall prevent or restrict the further exercise of that or any other right or remedy.

30. Compliance

30.1 The parties undertake:

(a) to comply in all respects with any applicable laws and regulations with respect on export controls and sanctions;

(b) not to violate (and represents and warrants that it has not violated):

(i) any applicable anti-corruption and anti-bribery laws;

(ii) any applicable money laundering laws, terrorist financing laws, or financial recordkeeping and reporting requirements; and

(iii) any applicable laws and regulations governing the importation and exportation of goods, equipment, technology, services, and information;

(c) to have and maintain in place effective controls that are sufficient to provide reasonable assurances that violations of applicable anti-corruption, anti-bribery, sanctions, import and export, terrorist financing and anti-money laundering laws and regulations will be prevented;

(d) to refrain from any behaviour that might constitute, in whole or in part, any of the offences referred to in Legislative Decree no. 231 of 8 June 2001;

(e) to notify each other, without undue delay, of any violation or potential violation of law committed by the parties and shall be responsible, indemnify and hold the other party harmless for any damages arising from any violations or potential violations of such laws.

31. Announcements

31.1 Subject to clause 31.2, neither party shall use the name or trade mark of the other party, or make, or permit any person to make, any public announcement, communication or circular ("**Announcement**") concerning the existence, subject matter or terms of this Agreement, the wider transactions contemplated by it, or the relationship between the parties, without the

prior written consent of the other parties (such consent not to be unreasonably withheld or delayed).

31.2 Where an Announcement is required by law, any relevant securities exchange, or by any court or other authority of competent jurisdiction, the party required to make the Announcement shall promptly notify the other party. The party concerned shall make all reasonable attempts to agree the contents of the

Announcement before making it.

32. Notices

32.1 Any notice to be given under this Agreement shall either be delivered by hand or sent by courier or via email.

Such communications must be sent to the respective Party at the following address (or to such other address that may be designated in writing by a Party to the other from time to time):

If to ICE

At the address set out above Attention of: Roger Viney
Email: r.viney@icepharma.com If to AMYLYX
At the address set out above Attention of: Tom Holmes
Email: amylyx_contracts@amylyx.com

A notice shall be deemed to have been served:

(a) if delivered by hand, at the time of delivery (with written confirmation of receipt);

(b) if sent by courier, at 9.00 am on the second Business Day after the envelope containing the same was delivered to the addressee;

(c) if sent by email, on the date sent e-mail of a PDF document (with confirmation of transmission) if sent during normal business hours of the party receiving the communication, and on the next business day if sent after normal business hours of the party receiving the communication.

32.2 This clause 32 does not apply to the service of any proceedings or other documents in any legal action or, where applicable, any arbitration or other method of dispute resolution.

32.3 A notice given under this Agreement is not valid if sent by email. For the avoidance of doubt, this clause 32 does not apply to day-to-day organisational communications between the parties, which may be sent by email.

33. Entire agreement

33.1 This Agreement and its annexes constitute the entire agreement between the parties with respect to the subject matter hereof, and supersedes and extinguishes all previous agreements, promises, assurances, warranties, representations and understandings between them, whether written or oral, relating to its subject matter. In particular, by means of this Agreement the parties terminate the RDS Agreement, that shall cease to regulate the relationship between the parties.

33.2 Each party acknowledges that in entering into this Agreement it does not rely on, and shall have no remedies for, any statement, representation, assurance or warranty (whether made innocently or negligently) that is not expressly set out in this Agreement. The only remedies available for any misrepresentation or breach of any representation or statement which was made prior to entry into this Agreement and which is expressly set out in this Agreement will be for breach of contract.

33.3 Each party agrees that it shall have no claim for negligent misrepresentation or misstatement based on any statement in this Agreement.

33.4 Nothing in this clause 33 shall limit or exclude any liability for fraud.

34. Governing law

34.1 This Agreement shall be governed, interpreted and constructed in accordance with the laws of England and Wales, without regard to the conflict of laws, rules, or principles thereof.

35. Dispute resolution and arbitration

35.1 All disputes arising out of or in connection with this Agreement shall be finally settled under the Rules of Arbitration of the International Chamber of Commerce by one or more arbitrators appointed in accordance with the said Rules.

35.2 The arbitration shall be conducted in English and the seat of the arbitration shall be London.

35.3 Unless the Parties expressly agree in writing to the contrary, the Parties undertake to keep confidential all awards in their arbitration, together with all materials in the proceedings created for the purpose of the arbitration and all other documents produced by another Party in the proceedings not otherwise in the public domain - save and to the extent that disclosure may be required of a Party by legal duty, to protect or pursue a legal right or to enforce or challenge an award in bona fide legal proceedings before a state court or other judicial authority.

Schedule 1 Prices

1) PRICE WITH RESPECT TO API DELIVERED UP TO 31 DECEMBER 2023

The Price for any Kg of API shall be equal to [***].

2) PRICE WITH RESPECT TO API DELIVERED AFTER 31 DECEMBER 2023 THROUGH 31 DECEMBER 2028

The Price of the API shall be calculated according to the table and the rules set out below

	Quantity per tier (kg)	**Total Quantity (kg)**	**Price per tier (US$)**
Minimum Vol.	[***]	[***]	[***]

Addition	[***]	[***]	[***]
Addition	[***]	[***]	[***]
Addition	[***]	[***]	[***]
Addition	[***]	[***]	[***]
Addition	[***]	[***]	[***]
Addition	[***]	[***]	[***]
Addition	[***]	[***]	[***]

i. The Price in case of purchase of up to [***] of API is equal to [***];

ii. In case of purchase of additional quantities of API, the Price indicated as from the second row of the third column of the table above shall apply for each additional quantity of up to [***], provided that the price of [***] shall apply to any quantity exceeding [***];

iii. The Price initially applied by ICE (the "**Provisional Price**") will be determined on the basis of the total quantity of API to be purchased by AMYLYX in a Calendar Year as forecasted and communicated to ICE in the Rolling Updated Forecast transmitted to ICE in December of each Calendar Year (the "**Forecasted Amount**"). An example of how the Provisional Price is determined is set out below (see Example A);

iv. At the end of each Calendar Year a reconciliation will be made by ICE between the Provisional Price calculated on the basis of the Forecasted Amount and the effective Price based on the actual quantities of API effectively purchased by AMYLYX and ICE will either issue an invoice or a credit note to AMYLYX on the basis of the results of

the said reconciliation. An example of how the reconciliation is made is set out below (see Example B);

v. Any credit note referred to under (iv) above shall be set-off directly by ICE against the amounts due by AMYLYX under the immediately subsequent invoice(s) to be issued by ICE under the Agreement, while any invoice referred to under (iv) above shall be paid by AMYLYX according to clause 6 of the Agreement.

A) Example of determination of the Provisional Price

If the Forecasted Amount in a given Calendar Year is equal to [***] of API, then the Provisional Price per kg initially invoiced by ICE throughout the relevant Calendar Year (subject to the reconciliation referred to under (iv) above) would be equal to [***], calculated as follows: [***].

B) Follow-up - Example of reconciliation

If AMYLYX effectively purchases in the relevant Calendar Year [***] of API, the Price would be equal to Provisional Price and, as such, no reconciliation would be needed.

If AMYLYX purchases a quantity which is lower or higher (as the case may be) than [***], then a reconciliation

shall be made in order to determine the Price.

By way of example, if AMYLYX purchases [***] (i.e., a quantity which is lower than [***]), the Price per Kg would be equal to [***], calculated as follows:

[***]

Under this scenario, ICE would issue an invoice for an amount equal to the difference between the price for a volume of [***] and that for [***] multiplied by the volume purchased, equal to [***], calculated as follows:

[***]

If AMYLYX, on the contrary, purchases [***] (i.e., a quantity which is higher than [***]), the Price per kg would be equal to [***], calculated as follows:

[***]

Under this scenario ICE would issue a credit note for an amount equal to the difference between the price for a volume of [***] and that for [***] multiplied by the volume purchased, equal to [***] calculated as follows:

[***]

"

Schedule 2 Initial Forecast

Delivery Month	**Aug-23**	**Sep-23**	**Oct-23**	**Nov-23**	**Dec-23**	**Jan-24**	**Feb-24**	**Mar-24**	**Apr-24**	**May-24**	**Jun-24**	**Jul-24**	**Aug-24**	**Sep-24**	**Oct-24**	**Nov-24**	**Dec-24**
Quantity Planned	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]	[***]
Delivered qty																	
PO		PO102762		PO102762	PO102762												

Schedule 3 Mutual Confidentiality Agreement

MUTUAL CONFIDENTIALITY AGREEMENT

[***]

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Joshua B. Cohen, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Amylyx Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    i. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    ii. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    iii. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    iv. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    i. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    ii. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 10, 2023

By: /s/ **Joshua B. Cohen**
Joshua B. Cohen
Co-Chief Executive Officer

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Justin B. Klee, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Amylyx Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    i. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    ii. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    iii. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    iv. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    i. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    ii. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 10, 2023

By: /s/ **Justin B. Klee**
Justin B. Klee
Co-Chief Executive Officer

Exhibit 31.3

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, James M. Frates, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Amylyx Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    i. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    ii. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    iii. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    iv. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    i. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    ii. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 10, 2023

By: /s/ **James M. Frates**
James M. Frates
Chief Financial Officer

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Amylyx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending June 30, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: August 10, 2023

By: /s/ **Joshua B. Cohen**
Joshua B. Cohen
Co-Chief Executive Officer

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Amylyx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending June 30, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: August 10, 2023

By: /s/ **Justin B. Klee**
Justin B. Klee
Co-Chief Executive Officer

Exhibit 32.3

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Amylyx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending June 30, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: August 10, 2023

By: /s/ **James M. Frates**
James M. Frates
Chief Financial Officer