

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): May 9, 2024

AMYLYX PHARMACEUTICALS, INC.

(Exact name of Registrant as Specified in Its Charter)

Delaware  
(State or Other Jurisdiction  
of Incorporation)

001-41199

(Commission File Number)

46-4600503  
(IRS Employer  
Identification No.)

43 Thorndike, St.,  
Cambridge, MA  
(Address of Principal Executive Offices)

02141  
(Zip Code)

Registrant's Telephone Number, Including Area Code: (617) 682-0917

Not Applicable

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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 Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

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.

**Item 2.02 Results of Operations and Financial Condition.**

On May 9, 2024, Amylyx Pharmaceuticals, Inc. (the "Company") issued a press release announcing its financial results for the fiscal quarter ended March 31, 2024. A copy of the press release is furnished as Exhibit 99.1 hereto.

The information provided in this Form 8-K, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference into any of the Company's filings under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

**Item 9.01 Financial Statements and Exhibits**

(d) Exhibits.

<b>Exhibit Number</b>	<b>Description</b>
99.1	<a href="#">Press Release of the Company, dated May 9, 2024</a>
104	Cover Page Interactive Data File (embedded with the Inline XBRL document).

**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.

**AMLYX PHARMACEUTICALS, INC.**

Date: May 9, 2024

By: /s/ James M. Frates

James M. Frates

Chief Financial Officer

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### Amylyx Pharmaceuticals Reports First Quarter 2024 Financial Results

- *Company plans to engage with FDA based on interim data from the Phase 2 HELIOS clinical trial demonstrating improvements in pancreatic function, glycemic control, and vision in participants with Wolfram syndrome treated with AMX0035; topline data from all 12 participants at Week 24 anticipated in fall of 2024*
- *Interim analysis from the ORION study of AMX0035 in progressive supranuclear palsy continues to be expected in mid-2025*
- *Amylyx expects to initiate clinical trial of AMX0114 in ALS in the second half of 2024*
- *Cash, cash equivalents and short-term investments of \$373.3 million at March 31, 2024 provides expected cash runway into 2026*
- *Management to host conference call and webcast today at 8:00 a.m. Eastern Time*

CAMBRIDGE, Mass. May 9, 2024 – [Amylyx Pharmaceuticals, Inc.](#) (Nasdaq: AMLX) (“Amylyx” or the “Company”) today reported financial results for the first quarter ended March 31, 2024.

“As an organization deeply committed to ending the suffering caused by neurodegenerative diseases, we took swift action in the first quarter of 2024 to focus our resources on key clinical and preclinical programs and build on the vital foundation we have laid to date as we drive toward our mission. We are focused on diseases where there is a high unmet need for better therapies and where there are well-defined measurable biomarkers, rigorous supporting preclinical data, and well-defined rationales for our therapy’s mechanism of action,” said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. “We anticipate multiple near-term catalysts for our pipeline, including data from our ongoing clinical trials of AMX0035 in Wolfram syndrome this fall and PSP in mid-2025. We are also excited to bring AMX0114, our potent antisense oligonucleotide targeting inhibition of calpain-2, to the clinic in ALS in the second half of 2024.”

#### Recent Corporate Updates:

- **Amylyx announced formal intention to remove RELYVRIO®/ALBRIOZA™ from the market based on topline results from the Phase 3 PHOENIX trial.** In April, the Company announced that it started a process with the U.S. Food and Drug Administration (FDA) and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO/ALBRIOZA for the treatment of ALS and remove the product from the market in the U.S. and Canada. This decision was informed by topline PHOENIX trial results, engagement with regulatory authorities, and discussions with the ALS community. As of April 4, 2024, RELYVRIO/ALBRIOZA is no longer available for new patients, and patients currently on therapy in the U.S. and Canada who, in consultation with their physician, wish to stay on treatment can be transitioned to a free drug program. Amylyx also announced a restructuring to focus the Company’s financial resources on key programs and extend expected cash runway into 2026, through anticipated data readouts for AMX0035 in Wolfram syndrome and PSP, and AMX0114 in ALS.
- **Interim data from the ongoing Phase 2 HELIOS clinical trial of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO, also known as ursodoxicoltaurine]) in eight adults living with Wolfram syndrome demonstrated encouraging improvement in pancreatic beta cell function, glycemic control, and vision.** Amylyx is planning to meet with FDA based on these interim data. Wolfram is a rare, progressive, genetic disease impacting approximately 3,000 people in the U.S. There are no approved treatment options for Wolfram. The majority of people with Wolfram carry mutations in the *WFS1* gene, which encodes a protein called wolframin that spans the membrane of the endoplasmic reticulum (ER). Wolfram is considered a prototypical ER stress disorder because of the clear link between *WFS1* mutations and ER stress. AMX0035, which targets ER stress and associated mitochondrial

dysfunction, has shown benefit in cellular models of the disease and a genetic animal model of the disease. The interim data from eight participants who have completed 24 weeks of treatment showed evidence of improvements in beta cell function and glycemic control, as measured by c-peptide and other markers of glucose metabolism, rather than the worsening typically expected with disease progression. All eight participants met prespecified responder criteria demonstrating either improvement or stabilization of disease according to both the Patient Reported Global Impression of Change (PGIC) and the Clinician Reported Global Impression of Change (CGIC) scales. The majority of participants reported some improvement in vision. AMX0035 was generally well-tolerated in all participants, and there have been no dropouts or discontinuations thus far. Amylyx anticipates reporting topline data from all 12 participants at Week 24 in the fall of 2024.

- **The Company hosted a virtual webcast with Dr. Fumihiko Urano to discuss interim data from the Phase 2 HELIOS clinical trial of AMX0035 in people living with Wolfram syndrome.** Dr. Urano is a leading expert in Wolfram syndrome, the Principal Investigator of the Phase 2 HELIOS clinical trial in Wolfram syndrome, and the Samuel E. Schechter Professor of Medicine in the Division of Endocrinology, Metabolism & Lipid Research at Washington University School of Medicine in St. Louis. A replay is available at <https://investors.amylyx.com>.
- **Data from an interim analysis of the ORION study of AMX0035 in PSP are expected in mid-2025.** ORION is a randomized, double-blind, placebo-controlled Phase 3 clinical trial designed to assess the efficacy, safety, and tolerability of AMX0035 in people living with PSP. 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. PSP is considered a tauopathy based on the strong genetic linkage of tau variants to the disease and presence of tau pathology in post-mortem brain samples. Variants in ER stress-linked genes have also been associated with the disease. In addition to demonstrating that AMX0035 targets ER stress and mitochondrial dysfunction, AMX0035 has been shown to significantly reduce cerebrospinal fluid (CSF) tau in a Phase 2, randomized, placebo-controlled trial in Alzheimer's disease. There are currently no approved therapies for the treatment of PSP, and the disease is reported to affect seven in 100,000 people worldwide.
- **The Company plans to file an investigational new drug (IND) application, then initiate a multiple ascending dose clinical trial of AMX0114 in people living with ALS in the second half of 2024.** Amylyx has completed IND-enabling studies of AMX0114, a potent antisense oligonucleotide targeting inhibition of calpain-2, a well-established target in a number of neurological diseases and a protease known to cleave many substrates including neurofilament, tau, and TDP43 proteins. Amylyx has observed rescue of cellular degeneration and neurofilament biology in multiple cellular experiments with AMX0114.
- **Bernhardt G. Zeiher, MD, FCCP, FACP, appointed to Amylyx Board of Directors.** Dr. Zeiher has spent more than 20 years in drug development at companies including Astellas Pharma, Pfizer, Eli Lilly and Company, and Merck and oversaw the approval of 15 new treatments that addressed people's unmet needs in serious diseases with few to no treatment options.

#### **Financial Results for the First Quarter Ended March 31, 2024**

**Net product revenue:** Net product revenue was \$88.6 million for the three months ended March 31, 2024, compared to net product revenue of \$71.4 million for the same period in 2023. During these periods, net product revenue was primarily related to units of RELYVRIO and ALBRIOZA sold in the U.S. and Canada, respectively. On April 4, 2024, the Company announced that it had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO/ALBRIOZA and remove the product from the market in the U.S. and Canada based on topline results from the Phase 3 PHOENIX trial.

**Cost of Sales:** Cost of sales were \$116.4 million in the three months ended March 31, 2024, compared to cost of sales of \$5.3 million for the same period in 2023. Cost of sales in the three months ended March 31, 2024 included non-cash charges of approximately \$110.5 million associated with the write-down of inventory and loss on firm purchase

commitments related to the Company's decision to voluntarily discontinue the marketing authorizations for RELYVRIO/ALBRIOZA in the U.S. and Canada. During these periods, cost of sales consisted of costs to procure, manufacture, and distribute RELYVRIO and ALBRIOZA.

**R&D Expenses:** Research and development expenses were \$36.6 million for the three months ended March 31, 2024, compared to \$24.2 million for the same period in 2023. The increase was primarily driven by an increase in personnel-related expenses due to added headcount to support research and development efforts, an increase in spending on the Phase 3 ORION study of AMX0035 in PSP, and an increase in preclinical development activities.

**SG&A Expenses:** Selling, general and administrative expenses were \$57.8 million for the three months ended March 31, 2024, compared to \$44.0 million for the same period in 2023. The increase was primarily driven by higher personnel-related expenses due to added headcount to support the Company's launch, commercialization initiatives, and operations as a public company, and other expenses.

**Net Loss:** Net loss for the three months ended March 31, 2024 was \$118.8 million, or \$1.75 on a fully diluted per share basis, compared to a net income of \$1.6 million, or \$0.02 on a fully diluted per share basis for the same period in 2023.

**Cash Position:** Cash, cash equivalents, and short-term investments were \$373.3 million at March 31, 2024, compared to \$371.4 million at December 31, 2023. The Company expects cash runway into 2026, which will allow the organization to deliver on key upcoming milestones, including anticipated data readouts from HELIOS (AMX0035 in Wolfram syndrome), ORION (AMX0035 in PSP), and its planned trial of AMX0114 in ALS.

### **Investor Conference Call Information**

Amylyx' management team will host a conference call and webcast today, May 9, 2024, at 8:00 a.m. ET to discuss financial results and provide an update on the business. To access the conference call, please dial +1 (877)-346-6112 (U.S. & Canada) or +1 (848)-280-6350 (international) at least 10 minutes prior to the start time and ask to be joined into the Amylyx Pharmaceuticals call. A live audio webcast of the call will be available under "Events and Presentations" in the Investor section of the Company's website, <https://investors.amylyx.com/news-events/events>. The webcast will be archived and available for replay for 90 days following the event.

### **Available Information**

We periodically provide other information for investors on our corporate website, <https://amylyx.com>, and our investor relations website, <https://investors.amylyx.com>. This includes press releases and other information about financial performance, information on corporate governance, and details related to our annual meeting of stockholders. We intend to use our website as a means of disclosing material non-public information and for complying with our disclosure obligations under Regulation FD. Accordingly, investors should monitor our website, in addition to following the Company's press releases, SEC filings, and public conference calls and webcasts.

### **About AMX0035**

AMX0035 is an oral, fixed-dose combination of sodium phenylbutyrate (PB) and taurursodiol (TURSO; also known as ursodoxicoltaurine outside of the U.S.). AMX0035 was designed to slow or mitigate neurodegeneration by targeting endoplasmic reticulum (ER) stress and mitochondrial dysfunction, two connected central pathways that lead to cell death and neurodegeneration. Preclinical studies have provided evidence that AMX0035 may reduce cell death and improve cellular function, also supporting the synergistic effect of AMX0035 compared to individual compounds. AMX0035 is being studied as a potential treatment in neurodegenerative diseases, including Wolfram syndrome and progressive supranuclear palsy (PSP).

### **About AMX0114**

AMX0114 is an antisense oligonucleotide designed to target the gene encoding calpain-2, a key contributor to the axonal (Wallerian) degeneration pathway. Axonal degeneration has been recognized as an important early contributor to the clinical presentation and pathogenesis of ALS and other neurodegenerative diseases. Calpain-2 has been implicated in the pathogenesis of ALS based on findings of elevated levels of calpain-2 and its cleavage products in postmortem ALS tissue, therapeutic benefit of calpain-2 modulation in animal models of ALS, and the role of calpain-2 in cleaving neurofilament, a broadly researched biomarker in ALS. Preclinical studies completed to date have shown that AMX0114 achieves potent, dose-dependent, and durable knockdown of *CAPN2* mRNA expression and calpain-2 protein levels in human motor neurons. Moreover, in preclinical efficacy studies, treatment with AMX0114 reduced extracellular neurofilament light chain levels following neurotoxic insult in induced pluripotent stem cell (iPSC)-derived human motor neurons, and improved survival of iPSC-derived human motor neurons harboring ALS-linked, pathogenic TDP-43 mutations.

### **About the HELIOS Trial**

The HELIOS trial ([NCT05676034](https://clinicaltrials.gov/ct2/show/study/NCT05676034)) is a 12-participant, open-label Phase 2 trial designed to study the effect of AMX0035 on safety and tolerability, and various measures of endocrinological, neurological, and ophthalmologic function in adult participants living with Wolfram syndrome.

### **About Wolfram Syndrome**

Wolfram syndrome is an autosomal recessive neurodegenerative disease characterized by childhood-onset diabetes, optic nerve atrophy, and neurodegeneration. Common manifestations of Wolfram include diabetes mellitus, optic nerve atrophy, central diabetes insipidus, sensorineural deafness, neurogenic bladder, and progressive neurologic difficulties. Literature suggests approximately 3,000 people are living with Wolfram syndrome in the United States. Genetic and experimental evidence suggests that endoplasmic reticulum (ER) dysfunction is a critical pathogenic component of Wolfram. The prognosis of Wolfram is poor, and many people with the disease die prematurely with severe neurological disabilities.

## **About the ORION Trial**

The ORION trial ([NCT06122662](https://clinicaltrials.gov/ct2/show/study/NCT06122662)) is a global, randomized, double-blind, placebo-controlled Phase 3 clinical trial designed to assess the efficacy, safety, and tolerability of AMX0035 compared to placebo in people living with progressive supranuclear palsy (PSP). ORION was designed and planned in collaboration with key global academic leaders, people living with PSP and their caregivers, and industry advocacy organizations.

## **About PSP**

Progressive supranuclear palsy (PSP) is a sporadic, rare, and adult-onset neurodegenerative disorder that affects walking and balance, eye movement, swallowing, and speech. People living with PSP have a life expectancy of six to eight years after initial diagnosis, and its epidemiology is similar to that of amyotrophic lateral sclerosis (ALS). PSP typically begins in late-middle age and rapidly progresses over time. The disease affects approximately seven in 100,000 people worldwide, and there are currently no disease-modifying therapies approved for the treatment of PSP.

PSP is characterized by abnormal tau inclusions and is consequently also known as a tauopathy. Similar to other neurodegenerative diseases, pathophysiologic changes underlying PSP are multifactorial, with several genetic and environmental factors likely contributing to tau dysfunction and aggregation.

Multiple pathways, including genetic mutations, endoplasmic reticulum (ER) stress, and the activation of unfolded protein response, mitochondrial dysfunction, and neuroinflammation have been implicated as contributors to tau dysfunction and aggregation.

## **About ALS**

Amyotrophic lateral sclerosis (ALS, also known as motor neuron disease) is a relentlessly progressive and fatal neurodegenerative disorder caused by motor neuron death in the brain and spinal cord. Motor neuron loss in ALS leads to deteriorating muscle function, the inability to move and speak, respiratory paralysis, and eventually, death. More than 90% of people with ALS have sporadic disease, showing no clear family history. ALS affects around 30,000 people in the U.S., and more than 30,000 people are estimated to be living with ALS in Europe (European Union and the United Kingdom). People living with ALS have a median survival of approximately two years from diagnosis.

## **About Amylyx Pharmaceuticals**

Amylyx Pharmaceuticals, Inc. is committed to supporting and creating more moments for the neurodegenerative disease community through the discovery and development of innovative new treatments. Amylyx is headquartered in Cambridge, Massachusetts. For more information, visit [amylyx.com](https://amylyx.com) and follow us on [LinkedIn](#) and [X](#), formerly known as Twitter. For investors, please visit [investors.amylyx.com](https://investors.amylyx.com).

## **Forward-Looking Statements**

Statements contained in this press release and related comments in our earnings conference call regarding matters that are not historical facts are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, the potential of AMX0035 (sodium phenylbutyrate and taurursodiol) as a treatment for Wolfram syndrome and PSP or other neurodegenerative diseases; expectations regarding the timing of the announcement of results from the Company’s Phase 3 ORION trial of AMX0035 for the treatment of PSP, and additional results from the Company’s Phase 2 HELIOS trial of AMX0035 for the treatment of Wolfram syndrome, including planned discussions with regulatory authorities related to the latter; the potential for AMX0114 as a treatment for ALS and the planned initiation of a trial evaluating AMX0114 in ALS; the availability of RELYVRIO/ALBRIOZA for patients currently taking RELYVRIO/ALBRIOZA; the timing and significance of learnings from the PHOENIX trial; the Company’s expectations regarding its financial performance; expectations regarding the contributions of the Company’s board of directors; and expectations regarding the Company’s cash runway and longer-term strategy. Any forward-looking statements in this press release and related comments in the Company’s earnings conference call are



based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. Risks that contribute to the uncertain nature of the forward-looking statements include: the success, cost, and timing of Amylyx' program development activities; Amylyx' ability to execute on its regulatory development plans and expectations regarding the timing of results from its planned data announcements and initiation of clinical studies; the risk that early-stage results may not reflect later-stage results; Amylyx' ability to fund operations, and the impact that global macroeconomic uncertainty, geopolitical instability, and public health events will have on Amylyx' operations, as well as the risks and uncertainties set forth in Amylyx' United States Securities and Exchange Commission (SEC) filings, including Amylyx' Annual Report on Form 10-K for the year ended December 31, 2023, and subsequent filings with the SEC. All forward-looking statements contained in this press release and related comments in our earnings conference call speak only as of the date on which they were made. Amylyx undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

**AMYLYX PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
**UNAUDITED**  
**(in thousands)**

	March 31, 2024	December 31, 2023
<b>Assets</b>		
Cash, cash equivalents and short-term investments	\$ 373,293	\$ 371,362
Accounts receivable, net	20,351	40,050
Inventories	—	83,280
Prepaid expenses and other current assets	16,890	14,931
Other assets	6,923	7,831
Total assets	<u>\$ 417,457</u>	<u>\$ 517,454</u>
<b>Liabilities and Stockholders' Equity</b>		
Accounts payable and accrued expenses	\$ 89,327	\$ 79,785
Other liabilities	3,694	4,237
Total liabilities	<u>93,021</u>	<u>84,022</u>
Stockholders' equity	324,436	433,432
Total liabilities and stockholders' equity	<u>\$ 417,457</u>	<u>\$ 517,454</u>

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

	Three Months Ended March 31,	
	2024	2023
Product revenue, net	\$ 88,643	\$ 71,428
Operating expenses:		
Cost of sales	5,945	5,283
Cost of sales - inventory impairment and loss on firm purchase commitments	110,461	—
Research and development	36,608	24,192
Selling, general and administrative	57,759	44,006
Total operating expenses	<u>210,773</u>	<u>73,481</u>
Loss from operations	(122,130)	(2,053)
Other income, net	3,579	3,456
(Loss) income before income taxes	(118,551)	1,403
Provision (benefit) for income taxes	242	(170)
Net (loss) income	<u>\$ (118,793)</u>	<u>\$ 1,573</u>
Net (loss) income per share		
Basic	\$ (1.75)	\$ 0.02
Diluted	\$ (1.75)	\$ 0.02
Weighted-average shares used in computing net (loss) income per share		
Basic	67,854,356	66,717,271
Diluted	67,854,356	70,863,665

## **Contacts**

### **Media**

Amylyx Media Team

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### **Investors**

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