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): April 10, 2024

AMYLYX PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**001-41199**	**46-4600503**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

43 Thorndike St., Cambridge, MA	**02141**
(Address of principal executive offices)	**(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 8.01 Other Events.

On April 10, 2024, Amylyx Pharmaceuticals, Inc. (the "Company") announced interim data from the ongoing Phase 2 HELIOS clinical trial of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO, also known as ursodoxicoltaurine]) in adults living with Wolfram syndrome, a rare, progressive genetic disease impacting approximately 3,000 people in the U.S. The interim data from eight participants who have completed 24 weeks of treatment demonstrated that AMX0035 had a clinically meaningful effect on key outcomes measuring the progression of diabetes, visual decline, and overall disease burden in adult participants living with Wolfram syndrome.

The interim analysis performed is based on a data cutoff as of March 5, 2024, which includes all participants who completed their Week 24 assessments as of the cutoff (n=8).

In this interim analysis of eight participants treated with AMX0035, increases were observed on average in the primary outcome of total C-peptide response (C-peptide AUC change from baseline) including in the 90-minute response at Week 24 (+15.6 ng*min/mL, 95% CI: [1.3, 30.0]). In Wolfram syndrome, progressive decline would have been expected on this measure. Additionally, seven out of eight participants demonstrated at least a 30-minute shorter time to peak C-peptide response. In Wolfram syndrome, a progressive increase in time to peak C-peptide response, indicating slower pancreatic response, and reduced total C-peptide response would have been expected.

The following includes additional key data from the interim analysis:

- Hemoglobin A1C (HbA1c) is a measure of glycosylated hemoglobin which serves as a metric of how well sugar levels are being controlled in the blood. HbA1c was reduced by 0.26% (SE: 0.15%) on average after 24-weeks of AMX0035 treatment with six out of eight participants showing improvement in their HbA1c. Many studies have associated reduced HbA1c with better clinical outcomes.
- All participants had continuous glucose monitoring in the study allowing for a rigorous measurement of the time in target glucose range. The absolute time in target glucose range improved on average by +7.1% (SE: 4.7%). Five out of eight participants had improvements in the time in target glucose range. Increased time in target glucose range is associated with better diabetic outcomes.
- Visual acuity was measured by the Snellen chart. Wolfram syndrome results in progressive optic nerve atrophy leading to relentless loss of both visual acuity and color vision, and eventually blindness. On average in HELIOS, visual acuity improved +0.05 -LogMAR (SE: 0.09) with five out of eight participants demonstrating some improvement in vision. Of those who improved, one participant changed from legally blind to legally sighted. Optical coherence tomography outcomes have not yet been assessed and will be included in final data analysis from HELIOS.
- All participants (8 out of 8) showed disease stability or improvement at Week 24, as measured by The Clinician Report Global Impression of Change (CGIC) and Patient Reported Global Impression of Change (PGIC). Improvement was noted by the CGIC in 62.5% of cases (5 out of 8) and by the PGIC for 75% of cases (6 out of 8) with the remainder reporting disease stability on both the CGIC and PGIC. These outcome measures are designed to report if the overall burden of disease has improved, stayed the same, or worsened from the clinician's or patient's perspective.
- The safety profile of AMX0035 in HELIOS was consistent with prior safety data. AMX0035 was generally well-tolerated. The majority of adverse events (AEs) were mild or moderate, and there were no serious AEs related to AMX0035 treatment. The most common AE was diarrhea.

Forward-Looking Statements

Statements contained in this Current Report on Form 8-K 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, Amylyx’ expectations regarding: interactions with regulatory authorities; the ongoing evaluation of AMX0035 in Wolfram syndrome, including that early-stage results may not reflect later-stage results and the timing for expected topline data in the HELIOS study; and the potential for AMX0035 to help people living with Wolfram syndrome. Any forward-looking statements in this Current Report on Form 8-K 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 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 Current Report on Form 8-K 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.

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: April 10, 2024

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