Amylyx Pharmaceuticals Reports First Quarter 2025 Financial Results

May 8, 2025

- *Pivotal Phase 3 LUCIDITY trial of avexitide, a potential first-in-class GLP-1 receptor antagonist with FDA breakthrough therapy designation, underway in post-bariatric hypoglycemia (PBH); completion of recruitment expected in 2025, with topline data anticipated in first half of 2026*
- *Data through Week 48 from the ongoing Phase 2 HELIOS trial of AMX0035 in Wolfram syndrome to be presented at the Joint Congress of the European Society for Pediatric Endocrinology (ESPE) and the European Society of Endocrinology (ESE) taking place from May 10-13*
- *Phase 1 LUMINA trial of AMX0114, an investigational, potent antisense oligonucleotide targeting knockdown of calpain-2, underway in people living with amyotrophic lateral sclerosis (ALS); early cohort data expected in 2025*
- *Cash, cash equivalents, and marketable securities of $204.1 million as of March 31, 2025; cash runway expected through the end of 2026*
- *Management to host conference call and webcast today at 8:00 a.m. Eastern Time*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 8, 2025-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial and business results for the first quarter ended March 31, 2025.

"During the first quarter, we made continued progress across our pipeline, including strong clinical execution of our pivotal Phase 3 LUCIDITY trial of avexitide in post-bariatric hypoglycemia. We remain focused on avexitide and continue to expect completion of enrollment in 2025 and topline data in the first half of 2026," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "We also made important progress in dosing the first patient in our Phase 1 LUMINA trial of AMX0114 in ALS, with early cohort data expected later this year. Additionally, we look forward to presenting new, long-term data from the Phase 2 HELIOS trial of AMX0035 in Wolfram syndrome in the coming days, and the topline data from the Phase 2b portion of the Phase 2b/3 ORION trial of AMX0035 are expected in the third quarter. With our momentum so far this year and current anticipated cash runway through the end of 2026, we remain confident in achieving our current goals and look forward to our upcoming milestones."

First Quarter and Recent Updates:

- **Amylyx began dosing in the pivotal Phase 3 LUCIDITY clinical trial of avexitide, a glucagon-like peptide-1 (GLP-1) receptor antagonist with FDA Breakthrough Designation, for the treatment of post-bariatric hypoglycemia (PBH).** In April 2025, the Company dosed the first participant for LUCIDITY, an approximately 75-participant, randomized, double-blind, placebo-controlled clinical trial designed to evaluate efficacy and safety of avexitide in participants with PBH following Roux-en-Y gastric bypass (RYGB) surgery at approximately 20 sites in the U.S. Amylyx presented the design of LUCIDITY in December 2024, and the poster is available on the "Publications" page of the Amylyx website. Avexitide has been previously evaluated in five PBH clinical trials showing consistent, dose-dependent effects, including statistically significant reductions in hypoglycemic events. Avexitide was generally well-tolerated, with a favorable safety profile replicated across clinical trials. With these consistent data, Amylyx believes avexitide has the potential to become the first approved therapy for PBH.
- **Amylyx began dosing in the Phase 1 LUMINA clinical trial of AMX0114, an investigational antisense oligonucleotide (ASO) targeting knockdown of calpain-2, for people living with amyotrophic lateral sclerosis (ALS).** In April 2025, the Company dosed the first participant for LUMINA, a multinational, randomized, double-blind, placebo-controlled, multiple ascending dose clinical trial designed to evaluate the safety and biological activity of AMX0114. The trial will also assess ALS biomarkers, including changes from baseline in neurofilament light (NfL) levels. Approximately 48 participants will be randomized 3:1 to receive AMX0114 or placebo by intrathecal administration once every four weeks, for up to four doses.
- **Amylyx closed its underwritten public offering of 19.7 million shares of its common stock in January 2025.** The net proceeds to Amylyx from this offering were approximately $65.5 million, extending the company's expected cash runway through the end of 2026.

Upcoming Expected Milestones:

- **Completion of enrollment for the pivotal Phase 3 LUCIDITY clinical trial of avexitide in PBH expected in 2025, with a data readout anticipated in the first half of 2026 and, if approved, commercial launch anticipated in 2027.** LUCIDITY is evaluating the FDA-agreed-upon primary outcome of reduction in the composite of Level 2 and Level 3 hypoglycemic events through Week 16 and has similar inclusion and exclusion criteria to previous Phase 2 trials of

avexitide in PBH.

- **Amylyx will share Week 48 data from the ongoing Phase 2 HELIOS trial of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO, also known as ursodoxicoltaurine]) in Wolfram syndrome at the Joint Congress of the European Society for Pediatric Endocrinology (ESPE) and the European Society of Endocrinology (ESE) on May 10-13 in Copenhagen, Denmark.** The poster presentation will be made available on the "Presentations" tab of the Amylyx website on Monday, May 12, 2025. Data from participants at Week 48 and ongoing discussions with the FDA will inform the design of a Phase 3 trial of AMX0035 in Wolfram syndrome.
- **Amylyx plans to present additional data from the Phase 2 PREVENT and Phase 2b trials of avexitide in PBH at the Endocrine Society's annual meeting (ENDO) 2025 on July 12-15 in San Francisco, CA.** The presentations will be made available on the "Presentations" tab of the Amylyx website following the conclusion of the sessions in accordance with ENDO's embargo policy.
- **Unblinded analysis of the Phase 2b portion of the Phase 2b/3 ORION trial evaluating AMX0035 for progressive supranuclear palsy (PSP) expected in the third quarter of 2025.** ORION is an operationally seamless Phase 2b/3 clinical trial in people living with PSP. The Phase 2b portion was fully enrolled in January 2025 with a total of 139 participants randomized. Efficacy and safety Phase 2b data from an unblinded analysis when all participants have reached Week 24 will be used to inform a go/no-go decision on the Phase 3 portion of the trial.
- **Early cohort data from the Phase 1 LUMINA clinical trial of AMX0114 in ALS expected in 2025.** LUMINA clinical trial sites were activated in Canada in the beginning of 2025 and are recruiting participants. Amylyx is working to open U.S. sites for screening, enrollment, and dosing.

Financial Results for the First Quarter Ended March 31, 2025

R&D Expenses: Research and development expenses for the first quarter of 2025 were $22.1 million, compared to $36.6 million for the same period in 2024. The decrease was primarily due to a decrease in spending on AMX0035 for the treatment of ALS, payroll and personnel-related costs, and a decrease in preclinical development activities. Research and development expenses include $1.8 million of stock-based compensation expense for the quarter, compared to $2.7 million of stock-based compensation expense for the same period in 2024.

SG&A Expenses: Selling, general, and administrative expenses for the first quarter of 2025 were $15.7 million, compared to $57.8 million for the same period in 2024. The decrease was primarily due to a decrease in payroll and personnel-related costs and a decrease in consulting, professional services, and other expenses. Selling, general, and administrative expenses include $5.0 million of stock-based compensation expense for the quarter, compared to $7.2 million for the same period in 2024.

Net Loss: Net loss for the three months ended March 31, 2025 was $35.9 million, or $0.42 per share, compared to net loss of $118.8 million, or $1.75 per share for the same period in 2024.

Cash Position: Cash, cash equivalents, and marketable securities were $204.1 million at March 31, 2025, compared to $176.5 million at December 31, 2024. Based on its current operating plans, Amylyx expects its cash runway to be through the end of 2026. The cash position at March 31, 2025 reflects payments of $6.0 million in the first quarter of 2025 related to product rebates and the settlement of purchase commitments for AMX0035 that were established prior to the voluntary discontinuation of sales of RELYVRIO®/ALBRIOZA™ inApril 2024. We recorded $1.4M of expense in the first quarter of 2025 related to these payments, with the remaining expense recorded in prior periods. The residual cash obligations related to the discontinuation of RELYVRIO/ALBRIOZA are $3.1 million, and the Company expects they will be paid through 2025.

Investor Conference Call Information
Amylyx' management team will host a conference call today, May 8, 2025, 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 (800)-836-8184 (U.S. & Canada) or +1 (646)-357-8785 (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/events-presentations. 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 Avexitide
Avexitide is an investigational, first-in-class glucagon-like peptide-1 (GLP-1) receptor antagonist that has been evaluated in five Phase 1 and Phase 2 clinical trials for post-bariatric hypoglycemia (PBH) and has also been studied in congenital hyperinsulinism (HI). The U.S. Food and Drug Administration (FDA) has granted avexitide Breakthrough Therapy Designation for both indications, Rare Pediatric Disease Designation in congenital HI, and Orphan Drug Designation for the treatment of hyperinsulinemic hypoglycemia (which includes PBH and congenital HI). Avexitide is designed to bind to the GLP-1 receptor on pancreatic islet beta cells and inhibit the effect of GLP-1 to mitigate hypoglycemia by decreasing insulin secretion and stabilizing blood glucose levels.

In PBH, excessive GLP-1 can lead to the hypersecretion of insulin and subsequent debilitating hypoglycemic events. In two Phase 2 PBH clinical trials, avexitide demonstrated highly statistically significant reductions in hypoglycemic events. These events can lead to autonomic and neuroglycopenic symptoms that can have a devastating impact on daily living.

About Post-Bariatric Hypoglycemia (PBH)
Post-bariatric hypoglycemia (PBH) is a condition that affects approximately 8% of people in the U.S., or approximately 160,000 people, who have undergone the two most common types of bariatric surgery, which include sleeve gastrectomy and Roux-en-Y gastric bypass (RYGB). PBH is thought to be caused by an excessive glucagon-like peptide-1 (GLP-1) response leading to hypoglycemia and impaired quality of life. PBH can cause debilitating hypoglycemic events associated with inadequate supply of glucose to the brain, known as neuroglycopenia. Clinical manifestations can include impaired cognition, loss of consciousness, and seizures. PBH is also associated with a high degree of disability that can result in major disruptions to independent living. There are no approved therapies for PBH.

About the LUCIDITY Trial
LUCIDITY (NCT06747468) is an approximately 75-participant, multicenter, randomized, double-blind, placebo-controlled Phase 3 clinical trial evaluating the efficacy and safety of avexitide in participants with PBH following Roux-en-Y gastric bypass (RYGB) surgery. The Phase 3 trial will be conducted at approximately 20 sites in the U.S. Participants will be randomized 3:2 to receive either 90 mg of avexitide subcutaneously once daily or placebo. The trial includes an up to six-week screening period, including a three-week run-in period, and a 16-week double-blind treatment period. Participants who complete the double-blind period will be eligible to enter an open-label extension (OLE) period with a duration of 32 weeks. The primary efficacy objective of LUCIDITY will evaluate the FDA-agreed upon primary outcome of reduction in the composite of Level 2 and Level 3 hypoglycemic events through Week 16. Safety and tolerability will also be evaluated.

About Amylyx Pharmaceuticals
At Amylyx, our mission is to usher in a new era of treating diseases with high unmet needs. Where others see challenges, we see opportunities that we pursue with urgency, rigorous science, and unwavering commitment to the communities we serve. We are currently focused on three investigational therapies across several neurodegenerative and endocrine diseases in which we believe they can make the greatest impact. For more information, visit amylyx.com and follow us on LinkedIn and X. For investors, please visit investors.amylyx.com.

Forward-Looking Statements
Statements contained in this press release 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: the potential of avexitide as a treatment for PBH; and expectations regarding the timing for recruitment completion and topline data readout of the Phase 3 LUCIDITY trial of avexitide in PBH and regarding the upcoming presentation of additional data from the Phase 2 PREVENT and Phase 2b trials of avexitide; 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 Week 48 data from the Phase 2 HELIOS trial of AMX0035 for the treatment of Wolfram syndrome; planned discussions with the FDA related to AMX0035 for the treatment of Wolfram syndrome; expectations regarding the timing of the announcement of interim results from the Company’s Phase 2b/3 ORION trial of AMX0035 for the treatment of PSP; the potential for AMX0114 as a treatment for ALS and the expected timeline for data readout of the Phase 1 LUMINA clinical trial; and Amylyx’ expectations regarding its financial performance, 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; 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, 2024, 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, except as required by law.

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

	March 31, 2025	December 31, 2024
Assets		
Cash, cash equivalents and marketable securities	$ 204,068	$ 176,501
Accounts receivable, net	300	447

Prepaid expenses and other current assets	11,776	12,484
Other assets	3,532	4,202
Total assets	$ 219,676	$ 193,634
Liabilities and Stockholders' Equity		
Accounts payable and accrued expenses	$ 16,877	$ 26,888
Other liabilities	1,382	1,981
Total liabilities	18,259	28,869
Stockholders' equity	201,417	164,765
Total liabilities and stockholders' equity	$ 219,676	$ 193,634

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

	Three Months Ended March 31,	
	2025	**2024**
Product revenue, net	$ —	$ 88,643
Operating expenses:		
Cost of sales	—	5,945
Cost of sales - inventory impairment and loss on firm purchase commitments	—	110,461
Research and development	22,119	36,608
Selling, general and administrative	15,684	57,759
Total operating expenses	37,803	210,773
Loss from operations	(37,803)	(122,130)
Other income, net	1,896	3,579
Loss before income taxes	(35,907)	(118,551)
Provision for income taxes	—	242
Net loss	$ (35,907)	$ (118,793)
Net loss per share — basic and diluted	$ (0.42)	$ (1.75)
Weighted-average shares used in computing net loss per share — basic and diluted	85,697,108	67,854,356

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