Amylyx Pharmaceuticals Reports Fourth Quarter and Full Year 2025 Financial Results

March 3, 2026

- *Recruitment of the pivotal Phase 3 LUCIDITY trial of avexitide in PBH is complete; continue to expect to randomize and dose last eligible participants in Q1 2026, with topline data anticipated in Q3 2026*
- *Cash runway expected to fund operations through potential avexitide commercialization and into 2028*
- *Management to host conference call and webcast today at 8:00 a.m. Eastern Time*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 3, 2026-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial and business results for the fourth quarter and full year ended December 31, 2025.

"2025 was a year of meaningful advancement for Amylyx's pivotal avexitide program in post-bariatric hypoglycemia, as well as progress across our broader pipeline," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "In 2026, our primary focus is on our Phase 3 LUCIDITY trial of avexitide in PBH. With recruitment of LUCIDITY complete, we are on track to complete enrollment this month and continue to expect topline data in Q3 2026. We designed LUCIDITY with the goal of replicating and building on the robust body of evidence demonstrating statistically significant reductions in hypoglycemic events observed in five prior clinical trials of avexitide in PBH. We continue to be encouraged by the high participant interest and broad engagement seen across the LUCIDITY trial sites."

"Looking ahead, we have a clear trajectory toward potentially delivering the first FDA-approved therapy to the PBH community. Supported by a cash runway extending into 2028, we remain focused on disciplined execution as we continue to actively prepare for a regulatory submission and build our commercial infrastructure to support potential commercialization in 2027," continued Mr. Cohen and Mr. Klee.

Fourth Quarter and Recent Updates:

- **Amylyx announced the selection of AMX0318, a novel glucagon-like peptide-1 (GLP-1) receptor antagonist for long-acting administration, as a development candidate for post-bariatric hypoglycemia (PBH) and other rare diseases.** AMX0318 was selected as a development candidate after demonstrating robust preclinical and chemical properties, including a favorable pharmacokinetic profile that may support long-acting administration, a robust chemical stability profile, strong *in vitro* potency, evidence of *in vivo* activity and tolerability, and high solubility. AMX0318 was identified through a research collaboration with Gubra A/S, a company specializing in peptide-based drug discovery and preclinical contract research services.

- **Amylyx presented early safety and tolerability data from Cohort 1 of its Phase 1 LUMINA trial of AMX0114 at the 36th International Symposium on ALS/MND (MNDA) in December 2025.** AMX0114 is an investigational antisense oligonucleotide (ASO) targeting calpain-2 with U.S. Food and Drug Administration (FDA) Fast Track Designation for the potential treatment of amyotrophic lateral sclerosis (ALS). AMX0114 was generally well-tolerated in LUMINA trial participants enrolled in Cohort 1 (n=12), with no treatment-related serious adverse events (SAEs).

Upcoming Expected Milestones:

- **Recruitment of the pivotal Phase 3 LUCIDITY trial of avexitide in PBH is complete. Amylyx continues to expect to randomize and dose the last eligible participants this month with topline data expected in Q3 2026, and, if approved, a commercial launch in 2027.** Avexitide is an investigational, first-in-class GLP-1 receptor antagonist with FDA Breakthrough Therapy Designation in PBH. LUCIDITY is a 16-week, multicenter, randomized, double-blind, placebo-controlled trial evaluating the efficacy and safety of avexitide in approximately 75 participants with PBH following Roux-en-Y gastric bypass (RYGB) surgery. 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. Participants who complete the 16-week double-blind period are eligible to enter a 32-week open-label extension (OLE) period.

- **Completion of enrollment of Cohort 2 (n=12) of the Phase 1 LUMINA clinical trial of AMX0114 in ALS is expected in March 2026 and presentation of biomarker data from Cohort 1 (n=12) is expected in the first half of 2026**. The LUMINA trial is a randomized, double-blind, placebo-controlled, multiple ascending dose clinical trial of AMX0114 in people living with ALS, with Cohort 1 investigating the first and lowest of four doses being evaluated in the trial. LUMINA is evaluating the safety, tolerability, pharmacokinetics, and pharmacodynamics of AMX0114 in people living with ALS and assessing both novel and broadly researched ALS biomarkers, including change from baseline in neurofilament light chain (NfL) levels.

- **Investigational New Drug (IND)-enabling studies for AMX0318 are underway with an IND filing targeted for 2027.** AMX0318 has completed extensive preclinical evaluation, including stability, solubility, potency, *in vivo* pharmacokinetics and pharmacodynamics, and *in vivo* tolerability studies.

Financial Results for the Fourth Quarter and Year Ended December 31, 2025

R&D Expenses: Research and development expenses for the fourth quarter of 2025 were $21.2 million, compared to $22.9 million for the same period in 2024, and $90.4 million for the year ended December 31, 2025, compared to $104.1 million for the year ended December 31, 2024. The decrease was primarily due to a decrease in spending on AMX0035 for the treatment of ALS and progressive supranuclear palsy (PSP). The decrease was offset by increased spending related to the clinical development of avexitide in PBH. Research and development expenses include $1.6 million and $7.0 million of stock-based compensation expense for the quarter and year ended December 31, 2025, respectively, compared to $1.8 million and $8.8 million of stock-based compensation expense for the quarter and year ended December 31, 2024, respectively.

SG&A Expenses: Selling, general, and administrative expenses for the fourth quarter of 2025 were $15.4 million, compared to $17.1 million for the same period in 2024 and $62.9 million for the year ended December 31, 2025, compared to $114.3 million for the year ended December 31, 2024. This decrease was primarily due to a decrease in consulting and professional services. Selling, general, and administrative expenses include $4.8 million and $20.7 million of stock-based compensation expense for the quarter and year ended December 31, 2025, respectively, compared to $5.0 million and $24.3 million of stock-based compensation expense for the quarter and year ended December 31, 2024, respectively.

Net Loss: Net loss for the three months ended December 31, 2025 was $33.0 million, or $0.30 per share, compared to net loss of $37.5 million, or $0.55 per share for the same period in 2024. Net loss for the year ended December 31, 2025 was $144.7 million, or $1.53 per share, compared with net loss of $301.7 million, or $4.43 per share for the year ended December 31, 2024.

Cash Position: Cash, cash equivalents, and short-term investments were $317.0 million at December 31, 2025, compared to $344.0 million at September 30, 2025. Based on its current operating plans, Amylyx expects its cash runway into 2028.

Investor Conference Call Information

Amylyx's management team will host a conference call today, March 3rd, 2026, 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 (888)-880-3330 (U.S. & Canada) or +1 (646)-357-8766 (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 is estimated to affect approximately 8% of people in the U.S. who have undergone the two most common types of bariatric surgery, sleeve gastrectomy and Roux-en-Y gastric bypass (approximately 160,000 people in the U.S.). 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 is being conducted at 21 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 four investigational therapies across several endocrine conditions and neurodegenerative 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’s expectations regarding: the potential of avexitide as a treatment for PBH; expectations regarding the timing for enrollment completion and topline data readout of the Phase 3 LUCIDITY trial of avexitide; expectations regarding timing for potential commercialization of avexitide; the potential for AMX0114 as a treatment for ALS, the expected timeline for cohort 1 biomarker data from the Phase 1 LUMINA clinical trial, and expectation for regulatory action; the therapeutic potential for AMX0318, the expected timeline for a potential IND submission for AMX0318; and Amylyx’s 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’s program development activities; Amylyx’s 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’s ability to fund operations, and the impact that global macroeconomic uncertainty, geopolitical instability, and public health events will have on Amylyx’s operations, as well as the risks and uncertainties set forth in Amylyx’s United States Securities and Exchange Commission (SEC) filings, including Amylyx’s Annual Report on Form 10-K for the year ended December 31, 2025, 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)

	December 31, 2025	December 31, 2024
Assets		
Cash, cash equivalents and marketable securities	$ 316,979	$ 176,501
Accounts receivable, net	88	447
Inventories	—	—
Prepaid expenses and other current assets	6,604	12,484
Other assets	8,974	4,202
Total assets	$ 332,645	$ 193,634
Liabilities and Stockholders’ Equity		
Accounts payable and accrued expenses	$ 21,429	$ 26,888
Other liabilities	5,957	1,981
Total liabilities	27,386	28,869
Stockholders’ equity	305,259	164,765
Total liabilities and stockholders' equity	$ 332,645	$ 193,634

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

	Three Months Ended December 31,		Year Ended December 31,	
	2025	2024	2025	2024
Product revenue, net	$ —	$ (665)	$ —	$ 87,371
Operating expenses:				
Cost of sales	—	—	—	5,953
Cost of sales - inventory impairment and loss on firm purchase commitments	—	—	—	118,680
Acquired in-process research and development	—	—	—	36,203
Research and development	21,213	22,892	90,404	104,084
Selling, general and administrative	15,392	17,097	62,887	114,331
Restructuring expenses	—	—	—	22,851
Total operating expenses	36,605	39,989	153,291	402,102
Loss from operations	(36,605)	(40,654)	(153,291)	(314,731)
Other income, net	3,652	2,473	8,602	12,595
Loss before income taxes	(32,953)	(38,181)	(144,689)	(302,136)
Provision (benefit) for income taxes	46	(635)	46	(393)
Net loss	$ (32,999)	$ (37,546)	$ (144,735)	$ (301,743)
Net loss per share - basic and diluted	$ (0.30)	$ (0.55)	$ (1.53)	$ (4.43)
Weighted-average shares used in computing net loss per share - basic and diluted	109,841,882	68,593,499	94,565,567	68,142,158

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