Amylyx Pharmaceuticals Reports Third Quarter 2025 Financial Results

November 6, 2025

- *Completion of recruitment for pivotal Phase 3 LUCIDITY trial of avexitide expected in Q1 2026, with topline data anticipated in Q3 2026*
- *Cash runway expected into 2028, through the potential approval and commercial launch of avexitide*
- *Management to host conference call and webcast today at 8:00 a.m. Eastern Time*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 6, 2025-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial and business results for the third quarter ended September 30, 2025.

"Entering the final months of 2025, we are focused on execution as we head toward a transformative year in 2026, with topline data expected from the pivotal Phase 3 LUCIDITY trial," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "We continue to advance our lead asset avexitide, an investigational, first-in-class inhibitor of GLP-1 receptor activity with FDA Breakthrough Therapy Designation. We continue to see high participant interest and broad engagement across LUCIDITY clinical trial sites, reflecting the significant unmet need for PBH across the U.S. Based on our most recent projections of the trial enrollment rate, we expect completion of recruitment in Q1 2026, with topline data expected in Q3 2026. Our previous estimates assumed a ramp in the enrollment rate at this stage of the trial, but we have seen more of a steady enrollment rate in the last few weeks. Timing remains unchanged for the potential launch of avexitide, if approved, and we are executing on plans to be launch ready in 2027."

"Additionally, we are excited to share early cohort data from the ongoing Phase 1 LUMINA trial of AMX0114 in people living with ALS at the 36th International Symposium on ALS/MND in December. Separately, we are advancing the clinical development of AMX0035 in Wolfram syndrome, and pending alignment with the FDA, we plan to initiate a focused, pivotal Phase 3 clinical trial in the second half of 2026. We expect to have cash runway into 2028, which funds us through important milestones, including the potential launch of avexitide, if approved. Our team remains focused on execution to deliver meaningful innovation for people living with endocrine and neurodegenerative diseases," continued Mr. Cohen and Mr. Klee.

Third Quarter and Recent Updates:

- **Amylyx announced the closing of its underwritten public offering, with net proceeds to Amylyx of approximately $191 million.** The proceeds extend the company's expected cash runway into 2028, through the commercial launch of avexitide anticipated in 2027, if approved.

- **Amylyx presented new exploratory analyses from the Phase 2 PREVENT and Phase 2b clinical trials of avexitide, an investigational, first-in-class inhibitor of GLP-1 receptor activity with FDA Breakthrough Therapy Designation, for the treatment of PBH at the Endocrine Society's annual meeting (ENDO 2025) in July 2025.** The Phase 2 PREVENT trial evaluated avexitide in PBH following Roux-en-Y gastric bypass (RYGB) surgery, and the Phase 2b trial evaluated avexitide in PBH following a variety of upper gastrointestinal surgeries, including RYGB, sleeve gastrectomy, esophagectomy, Nissen fundoplication, and gastrectomy. In the Phase 2b trial, avexitide 90 mg once daily, the dose being evaluated in the pivotal Phase 3 LUCIDITY trial, led to a 64% least-squares mean reduction (p=0.0031) vs. baseline in the composite rate of Level 2 and Level 3 hypoglycemic events in PBH, with more than half of the participants experiencing no events during the treatment period. The 45 mg twice daily, 30 mg twice daily, and 60 mg once daily dose regimens all likewise demonstrated consistent reductions in composite rate of Level 2 and Level 3 hypoglycemic events. New pharmacokinetic and pharmacodynamic data were also presented, demonstrating continuous pharmacologic activity of the 90 mg once daily dose regimen for a 24-hour period. Avexitide was generally well tolerated, with a favorable safety profile replicated across clinical trials.

- **Amylyx is advancing the clinical development of AMX0035 in Wolfram syndrome and, pending alignment with the FDA, planning to initiate a focused, pivotal Phase 3 trial in the second half of 2026.** Amylyx previously reported positive topline results from the Company's Phase 2 HELIOS trial, an open-label study of 12 adult participants. At Week 24, stabilization or improvement was demonstrated across all key clinical measures, including pancreatic function, glycemic control, and vision. Long-term Week 48 data demonstrated that treatment with AMX0035 led to continued sustained stabilization or improvement.

- **Amylyx' research collaboration with Gubra A/S to develop a novel long-acting GLP-1 receptor antagonist continues to show encouraging proof-of-concept data.** Amylyx expects to make a decision on a potential development candidate in the next few months, and pending a candidate nomination, expects to initiate IND-enabling studies.

Upcoming Expected Milestones:

- **Completion of recruitment for the pivotal Phase 3 LUCIDITY trial is expected in Q1 2026, with topline data expected in Q3 2026 based on the most recent projections.** The Company continues to see high participant interest and broad engagement across clinical trial sites, reflecting the unmet need for PBH across the U.S. If approved, commercial launch is anticipated in 2027. LUCIDITY is a multicenter, randomized, double-blind, placebo-controlled Phase 3 clinical trial evaluating the efficacy and safety of avexitide in approximately 75 participants following RYGB surgery at approximately 20 sites in the U.S. 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.
- **Fully enrolled cohort 1 (n=12) of the Phase 1 LUMINA clinical trial of AMX0114 in ALS in September 2025 and expect to present early cohort data at the 36th International Symposium on ALS/MND held from December 5-7.** LUMINA is a multinational, randomized, double-blind, placebo-controlled, multiple ascending dose clinical trial designed to evaluate the safety and biological activity of AMX0114. 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. The trial will also assess ALS biomarkers, including changes from baseline in neurofilament light levels. Based on biomarker collection and analysis timelines, the Company anticipates biomarker data will be available in the coming months and expects to present these at a medical meeting in the first half of 2026. In June 2025, Amylyx announced that the FDA granted Fast Track designation to AMX0114.

Financial Results for the Third Quarter Ended September 30, 2025

R&D Expenses: Research and development expenses for the third quarter of 2025 were $19.9 million, compared to $21.2 million for the same period in 2024. The decrease was primarily due to decreases in spending on AMX0035 for the treatment of PSP and ALS. The decrease was offset by increased spending related to the clinical development of avexitide in PBH. Research and development expenses include $1.6 million of stock-based compensation expense for the quarter, compared to $1.8 million of stock-based compensation expense for the same period in 2024.

SG&A Expenses: Selling, general, and administrative expenses for the third quarter of 2025 were $16.2 million, compared to $17.8 million for the same period in 2024. The decrease was primarily due to a decrease in consulting, professional services, and other expenses. Selling, general, and administrative expenses include $5.4 million of stock-based compensation expense for the quarter, compared to $5.0 million for the same period in 2024.

Net Loss: Net loss for the three months ended September 30, 2025 was $34.4 million, or $0.37 per share, compared to net loss of $72.7 million, or $1.07 per share for the same period in 2024.

Cash Position: Cash, cash equivalents, and marketable securities were $344.0 million at September 30, 2025, compared to $180.8 million at June 30, 2025. The Company's cash position reflects net proceeds of approximately $191 million following the Company's public offering in September 2025. Based on its current operating plans, Amylyx expects its cash runway to extend into 2028.

Investor Conference Call Information

Amylyx' management team will host a conference call today, November 6, 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 (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 inhibitor of glucagon-like peptide-1 (GLP-1) receptor activity 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 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; expectations regarding the timing for recruitment completion and topline data readout of the Phase 3 LUCIDITY trial of avexitide; expectations regarding timing for potential commercialization of avexitide; expectations regarding the potential of AMX0035 (sodium phenylbutyrate and taurursodiol) as a treatment for Wolfram syndrome and planned initiation of a pivotal Phase 3 trial pending alignment with the FDA; the potential for AMX0114 as a treatment for ALS, the expected timeline for data readout of the Phase 1 LUMINA clinical trial, and expectation for regulatory action; expectations regarding the collaboration with Gubra A/S for development of a novel long-acting GLP-1 receptor antagonist, including and the potential next steps; 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)

	September 30, 2025	December 31, 2024
Assets		
Cash, cash equivalents and marketable securities	$ 343,990	$ 176,501
Accounts receivable, net	132	447
Prepaid expenses and other current assets	9,261	12,484
Other assets	9,358	4,202

Total assets	$ 362,741	$ 193,634
Liabilities and Stockholders' Equity		
Accounts payable and accrued expenses	$ 24,810	$ 26,888
Other liabilities	5,935	1,981
Total liabilities	30,745	28,869
Stockholders' equity	331,996	164,765
Total liabilities and stockholders' equity	$ 362,741	$ 193,634

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Product revenue, net	$ —	$ 416	$ —	$ 88,036
Operating expenses:				
Cost of sales	—	—	—	5,953
Cost of sales - inventory impairment and loss on firm purchase commitments	—	809	—	118,680
Acquired in-process research and development	—	36,203	—	36,203
Research and development	19,855	21,237	69,191	81,192
Selling, general and administrative	16,172	17,828	47,495	97,234
Restructuring expenses	—	—	—	22,851
Total operating expenses	36,027	76,077	116,686	362,113
Loss from operations	(36,027)	(75,661)	(116,686)	(274,077)
Other income, net	1,641	2,957	4,950	10,122
Loss before income taxes	(34,386)	(72,704)	(111,736)	(263,955)
Provision for income taxes	—	—	—	242
Net loss	$ (34,386)	$ (72,704)	$ (111,736)	$ (264,197)
Net loss per share — basic and diluted	$ (0.37)	$ (1.07)	$ (1.25)	$ (3.89)
Weighted-average shares used in computing net loss per share — basic and diluted	93,332,930	68,091,446	89,417,505	67,990,613

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