Amylyx Pharmaceuticals Reports First Quarter 2026 Financial Results

May 7, 2026

- *Topline data readout from Phase 3 LUCIDITY clinical trial of avexitide in post-bariatric hypoglycemia on track; 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)--May 7, 2026-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial and business results for the first quarter ended March 31, 2026.

"With enrollment complete in the pivotal Phase 3 LUCIDITY clinical trial, we have a clear line of sight to the anticipated Q3 topline data readout, bringing us one step closer to the potential of delivering the first approved therapy for the post-bariatric hypoglycemia community," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "We understand the devastating daily burden of this condition and are operating with a sense of urgency to advance our program. We have initiated regulatory and commercial readiness activities to help ensure we are positioned to move swiftly following LUCIDITY topline data. Supported by an expected cash runway extending into 2028, we are executing with focus and discipline as we work to bring this treatment to the PBH community in 2027, if approved."

First Quarter and Recent Updates:

- **Amylyx completed enrollment for the pivotal Phase 3 LUCIDITY clinical trial of avexitide, an investigational, first-in-class glucagon-like peptide-1 (GLP-1) receptor antagonist with U.S. Food and Drug Administration (FDA) Breakthrough Therapy Designation in post-bariatric hypoglycemia (PBH), in March 2026.** LUCIDITY enrolled 78 participants and is a 16-week, multicenter, randomized, double-blind, placebo-controlled trial evaluating the efficacy and safety of avexitide in adults with PBH following Roux-en-Y gastric bypass (RYGB) surgery. Participants who complete the 16-week double-blind period are eligible to enter a 32-week open-label extension (OLE) period.
- **Amylyx announced the initiation of an Expanded Access Program (EAP) for the use of avexitide to treat U.S. adults with PBH following RYGB surgery in May 2026.** Initial eligible patients include individuals who have completed the pivotal Phase 3 LUCIDITY clinical trial and participants in a prior trial of avexitide in PBH following RYGB surgery.
- **Amylyx completed enrollment of Cohort 2 (n=12) of the Phase 1 LUMINA clinical trial of AMX0114, an investigational antisense oligonucleotide (ASO) targeting calpain-2 with FDA Fast Track Designation for the potential treatment of amyotrophic lateral sclerosis (ALS), in March 2026.** The LUMINA trial is a randomized, double-blind, placebo-controlled, multiple ascending dose clinical trial of AMX0114 in people living with ALS. 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.

Upcoming Expected Milestones:

- **Topline data readout for Phase 3 LUCIDITY clinical trial of avexitide in PBH is on track and anticipated in the third quarter of 2026**. If approved, commercial launch of avexitide is 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. LUCIDITY was informed by data from five prior PBH clinical trials of avexitide showing consistent effects, most notably statistically significant reductions in Level 2 and Level 3 hypoglycemic events. Avexitide was generally well-tolerated, with a favorable safety profile replicated across previous clinical trials.
- **Presentation of early biomarker data from Cohort 1 (n=12) of the Phase 1 LUMINA clinical trial of AMX0114 in ALS is expected at the 2026 European Network to Cure ALS (ENCALS) Annual Meeting in June 2026**. Cohort 1 of LUMINA is investigating the first and lowest of four doses being evaluated in the trial. The data are expected to provide initial information about the levels of the ALS biomarkers being assessed from the first dose in the LUMINA trial. The Company previously presented early safety and tolerability data from Cohort 1 of LUMINA showing AMX0114 was generally well-tolerated, with no treatment-related serious adverse events (SAEs).
- **Investigational New Drug (IND)-enabling studies for AMX0318, a novel GLP-1 receptor antagonist for long-acting administration to treat PBH and other rare diseases, are underway with an IND filing targeted for 2027.** 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 (Gubra), a company specializing in peptide-based drug discovery and preclinical contract research services.

Financial Results for the First Quarter Ended March 31, 2026

R&D Expenses: Research and development expenses for the first quarter of 2026 were $27.6 million, compared to $22.1 million for the same period in 2025. The increase was primarily due to an increase in spending related to the clinical development of avexitide in PBH. Milestone payments totaling $4.0 million to Gubra were also recognized following the selection and handover of AMX0318 as a development candidate for PBH and other rare diseases. The increase was offset primarily by decreased spending related to AMX0035 for the treatment of progressive supranuclear palsy (PSP). Research and development expenses include $1.8 million of stock-based compensation expense for the quarter ended March 31, 2026, compared to $1.8 million of stock-based compensation expense for the quarter ended March 31, 2025.

SG&A Expenses: Selling, general, and administrative expenses for the first quarter of 2026 were $16.2 million, compared to $15.7 million for the same period in 2025. This increase was primarily due to an increase in consulting and professional services. Selling, general, and administrative expenses include $4.4 million of stock-based compensation expense for the quarter ended March 31, 2026, compared to $5.0 million of stock-based compensation expense for the quarter ended March 31, 2025.

Net Loss: Net loss for the three months ended March 31, 2026 was $41.3 million, or $0.37 per share, compared to net loss of $35.9 million, or $0.42 per share, for the same period in 2025.

Cash Position: Cash, cash equivalents, and short-term investments were $279.8 million at March 31, 2026, compared to $317.0 million at December 31, 2025. Based on its current operating plans, Amylyx expects a cash runway into 2028.

Investor Conference Call Information

Amylyx's management team will host a conference call today, May 7, 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

Amylyx periodically provides other information for investors on the Company's corporate website, https://amylyx.com, and the Company's 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. Amylyx intends to use its website as a means of disclosing material non-public information and for complying with its disclosure obligations under Regulation FD. Accordingly, investors should monitor Amylyx's 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). In PBH, an exaggerated GLP-1 response leads to excessive insulin secretion, resulting in recurrent hypoglycemic events. Avexitide is a GLP-1 receptor antagonist designed to competitively bind to the GLP-1 receptor on pancreatic islet beta cells and inhibit the exaggerated GLP-1-driven insulin response characteristic of PBH, reducing inappropriate insulin secretion and stabilizing blood glucose levels. In two Phase 2 PBH clinical trials, avexitide demonstrated highly statistically significant reductions in hypoglycemic events.

About Post-Bariatric Hypoglycemia (PBH)

PBH is a chronic metabolic 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 driven by an exaggerated glucagon-like peptide-1 (GLP-1) response, primarily in response to food intake, leading to persistent, recurrent, and often debilitating rapid drops in blood glucose, known as hypoglycemia. The American Diabetes Association (ADA) recognizes hypoglycemia as a potential medical emergency because low blood glucose levels can compromise the body's ability to maintain essential physiologic processes. In addition, hypoglycemia in the context of PBH may manifest as neuroglycopenia – an inadequate supply of glucose to the brain – which can cause confusion, cognitive dysfunction, loss of consciousness, and seizures. PBH can be associated with substantial disability, compromising safety, disrupting independent living, and affecting nutritional status and overall quality of life. Despite the substantial burden, there are currently no FDA-approved therapies for PBH.

About the LUCIDITY Trial

LUCIDITY (NCT06747468) is a 78-participant, multicenter, randomized, double-blind, placebo-controlled Phase 3 clinical trial evaluating the efficacy and safety of avexitide in participants with PBH following RYGB surgery. The Phase 3 trial is being conducted at 21 sites in the U.S. Participants were 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, a 16-week double-blind treatment period, and an open-label extension (OLE) period with a duration of 32 weeks. The primary efficacy objective of LUCIDITY is to 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 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; the timing for the topline data readout and completion of the Phase 3 LUCIDITY clinical trial of avexitide; the timing for potential commercialization of avexitide; the expected enrollment of the Expanded Access Program for avexitide; the potential for AMX0114 as a treatment for ALS; the expected timeline and announcement for Cohort 1 biomarker data from the Phase 1 LUMINA clinical trial, and enrollment and progress of the LUMINA trial; the therapeutic potential of AMX0318 and the expected timeline for a potential IND submission; and the potential benefits of expedited program and orphan designations held by Amylyx; and 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)

	March 31, 2026	December 31, 2025
Assets		
Cash, cash equivalents and marketable securities	$ 279,768	$ 316,979
Prepaid expenses and other current assets	5,256	6,692
Other assets	8,579	8,974
Total assets	$ 293,603	$ 332,645
Liabilities and Stockholders’ Equity		
Accounts payable and accrued expenses	$ 14,811	$ 21,429
Other liabilities	5,631	5,957
Total liabilities	20,442	27,386
Stockholders’ equity	273,161	305,259
Total liabilities and stockholders' equity	$ 293,603	$ 332,645

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
UNAUDITED

(in thousands, except share and per share data)

	Three Months Ended March 31,	
	2026	**2025**
Operating expenses:		
Research and development	$ 27,610	$ 22,119
Selling, general and administrative	16,169	15,684
Total operating expenses	43,779	37,803
Loss from operations	(43,779)	(37,803)
Other income, net	2,495	1,896
Net loss	$ (41,284)	$ (35,907)
Net loss per share — basic and diluted	$ (0.37)	$ (0.42)
Weighted-average shares used in computing net loss per share — basic and diluted	110,563,360	85,697,108

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