Amylyx Pharmaceuticals Reports Second Quarter 2026 Financial Results

August 6, 2026

- *Last participant completed final study visit in the 16-week double-blind period of the pivotal Phase 3 LUCIDITY trial of avexitide in post-bariatric hypoglycemia; topline data on track and anticipated in late August or early September 2026*
- *Cash runway expected to fund operations into 2028*
- *Management to host conference call and webcast today at 8:00 a.m. Eastern Time*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 6, 2026-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial and business results for the second quarter ended June 30, 2026.

"With the last participant's final study visit recently completed, we are eagerly anticipating the expected topline data readout of the pivotal Phase 3 LUCIDITY trial in post-bariatric hypoglycemia in late August or early September," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "In parallel, we are continuing to advance our NDA readiness and our pre-commercial work to support a potential submission and commercial launch of avexitide in 2027, if approved. At ENDO in June 2026, we activated our disease state education campaign, 'Uncover the Mystery of Post-Bariatric Hypoglycemia,' designed to increase awareness and understanding action around PBH. We look forward to sharing updates as we work toward potentially delivering the first FDA-approved therapy for the PBH community."

Second Quarter and Recent Updates:

- **The last participant has recently completed the final study visit in the 16-week double-blind period of the pivotal Phase 3 LUCIDITY clinical trial of avexitide, an investigational, first-in-class glucagon-like peptide-1 (GLP-1) receptor antagonist that has received U.S. Food and Drug Administration (FDA) Breakthrough Therapy Designation in post-bariatric hypoglycemia (PBH).** LUCIDITY enrolled 78 participants and is a 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 period.
- **Amylyx presented two posters at the Endocrine Society's Annual Meeting (ENDO 2026) in June 2026.** The first poster characterized the clinical, economic, and humanistic burden of PBH in the U.S. on both a patient and systemic level, evaluating how Level 2 and 3 hypoglycemic events in PBH impact healthcare utilization, productivity, and overall cost. The second poster described participant-centric trial design considerations for the LUCIDITY trial of avexitide in PBH.
- **Amylyx announced the initiation of a U.S. Expanded Access Program for the use of avexitide to treat adults with PBH following RYGB surgery in May 2026.** Initial eligible patients include individuals who have completed the LUCIDITY trial or participated in a prior trial of avexitide in PBH following RYGB surgery.
- **Amylyx entered into a second research collaboration with Gubra A/S in July 2026 to identify potential development candidates for a rare endocrine disease of high unmet need.**
- **Amylyx presented data from its first-in-human, dose-ranging Phase 1 LUMINA clinical trial of AMX0114, an investigational antisense oligonucleotide (ASO) targeting calpain-2 for the potential treatment of amyotrophic lateral sclerosis (ALS), at the 2026 European Network to Cure ALS (ENCALS) Annual Meeting in June 2026.** AMX0114 showed no drug-related serious adverse events (AEs) and no serious neurological AEs in Cohort 1, supporting continued evaluation at higher dose levels. Cohort 1 evaluated the lowest of four planned dose levels. Cohorts 1 (12.5 mg) and 2 (25 mg) are fully enrolled, and Cohort 3 (50 mg) is currently enrolling.
- **Amylyx announced the peer-reviewed publication of Week 24 and Week 48 results from the Phase 2 open-label HELIOS clinical trial of AMX0035 in adults living with Wolfram syndrome in *The Journal of Clinical Investigation* in May 2026.** The results reinforced consistency of observed stabilization or improvement across multiple outcomes related to disease progression, including pancreatic beta cell function, glycemic control, vision, and overall symptom burden. AMX0035 was generally well-tolerated, consistent with previously presented safety data.
- **Amylyx also presented longer-term Week 96 data from HELIOS in June 2026**. At Week 96, measures of pancreatic function and other measures of glycemic control were stable or improved relative to baseline in most participants. Visual acuity and patient- and clinician-reported outcomes showed patterns consistent with disease stabilization with interpretation limited by the open-label, single-arm design and small sample size (n=9 participants with data at Week 96). The safety profile of AMX0035 in HELIOS at Week 96, Week 48, and Week 24 were generally consistent with prior safety data from

the studies of AMX0035. Nearly all AEs were mild or moderate, and there were no serious AEs related to AMX0035 treatment. The Company continues to work with the FDA on a Phase 3 trial in Wolfram syndrome.

Upcoming Expected Milestones:

- **Topline data readout for the Phase 3 LUCIDITY clinical trial of avexitide in PBH is on track and anticipated in late August or early September 2026.** 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. The design of the LUCIDITY trial was informed by data from five prior clinical trials of avexitide in PBH, which showed consistent effects, most notably statistically significant reductions in Level 2 and Level 3 hypoglycemic events. Across previous clinical trials, avexitide was generally well-tolerated with a favorable safety profile. If approved, commercial launch of avexitide is anticipated in 2027.
- **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, a company specializing in peptide-based drug discovery and preclinical contract research services.

Financial Results for the Second Quarter Ended June 30, 2026

R&D Expenses: Research and development expenses for the second quarter of 2026 were $23.8 million, compared to $27.2 million for the same period in 2025. The decrease was primarily due to a decrease in spending related to AMX0035 for the treatment of progressive supranuclear palsy. The decrease was offset primarily by an increase in spending related to the clinical development of avexitide in PBH and other costs related to avexitide. Research and development expenses include $2.5 million of stock-based compensation expense for the quarter ended June 30, 2026, compared to $2.0 million of stock-based compensation expense for the quarter ended June 30, 2025.

SG&A Expenses: Selling, general, and administrative expenses for the second quarter of 2026 were $21.9 million, compared to $15.6 million for the same period in 2025. This increase was primarily due to higher legal expenses and increased investment in commercial strategic initiatives. Selling, general, and administrative expenses include $5.8 million of stock-based compensation expense for the quarter ended June 30, 2026, compared to $5.4 million of stock-based compensation expense for the quarter ended June 30, 2025.

Net Loss: Net loss for the three months ended June 30, 2026 was $43.4 million, or $0.39 per share, compared to net loss of $41.4 million, or $0.46 per share, for the same period in 2025.

Cash Position: Cash, cash equivalents, and short-term investments were $250.8 million at June 30, 2026, compared to $279.8 million at March 31, 2026. 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, August 6, 2026, at 8:00 a.m. ET. 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 AMX0114

AMX0114 is an investigational antisense oligonucleotide (ASO) with U.S. Food and Drug Administration (FDA) Fast Track designation for the potential treatment of ALS. AMX0114 targets calpain-2 (*CAPN2*), a calcium-activated protease that is one of the fundamental drivers of axonal degeneration and consequent disease progression in ALS. In preclinical studies, treatment with AMX0114 resulted in potent, dose-dependent, and durable reduction in *CAPN2* mRNA and calpain-2 protein levels in disease-relevant cell models of axonal degeneration. This translated to improved neuronal survival, including in a model of TDP-43 ALS, and reductions in extracellular neurofilament light (NfL) levels across multiple disease models and paradigms of neuronal injury. AMX0114 was generally well-tolerated in LUMINA trial participants enrolled in Cohort 1 (n=12), with no treatment-related serious adverse events (SAEs).

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 therapeutic 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, if approved; the expected enrollment and progress of the Expanded Access Program for avexitide; the therapeutic potential for AMX0114 as a treatment for ALS; the expected enrollment and progress of the LUMINA trial; the therapeutic potential of AMX0318 and the expected timeline for a potential IND submission; the therapeutic potential of AMX0035 as a treatment for Wolfram syndrome and plans for a Phase 3 trial; the potential benefits of expedited program and orphan designations held by Amylyx; the potential benefits of the research collaborations with Gubra A/S; 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)

	June 30, 2026	December 31, 2025
Assets		
Cash, cash equivalents and marketable securities	$ 250,760	$ 316,979
Prepaid expenses and other current assets	5,578	6,692
Other assets	8,226	8,974
Total assets	$ 264,564	$ 332,645
Liabilities and Stockholders' Equity		
Accounts payable and accrued expenses	$ 20,142	$ 21,429
Other liabilities	5,255	5,957
Total liabilities	25,397	27,386
Stockholders' equity	239,167	305,259
Total liabilities and stockholders' equity	$ 264,564	$ 332,645

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 23,768	$ 27,217	$ 51,379	$ 49,336
Selling, general and administrative	21,925	15,640	38,093	31,324
Total operating expenses	45,693	42,857	89,472	80,660
Loss from operations	(45,693)	(42,857)	(89,472)	(80,660)
Other income, net	2,270	1,414	4,765	3,310
Net loss	$ (43,423)	$ (41,443)	$ (84,707)	$ (77,350)
Net loss per share — basic and diluted	$ (0.39)	$ (0.46)	$ (0.76)	$ (0.88)
Weighted-average shares used in computing net loss per share — basic and diluted	111,196,883	89,138,568	110,881,872	87,427,345

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