Amylyx Pharmaceuticals Reports Second Quarter 2025 Financial Results

August 7, 2025

- *Completion of recruitment for pivotal Phase 3 LUCIDITY trial of avexitide in PBH expected in 2025, with topline data anticipated in first half of 2026*
- *Cash, cash equivalents, and marketable securities of $180.8 million as of June 30, 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)--Aug. 7, 2025-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial and business results for the second quarter ended June 30, 2025.

"As we look ahead to the second half of the year and into 2026, we remain encouraged by the strength of our pipeline and the continued momentum towards our clinical milestones," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "We expect to complete recruitment in the pivotal Phase 3 LUCIDITY trial of avexitide in 2025, with topline data anticipated in the first half of 2026. In the third quarter, we expect to share an unblinded analysis from the Phase 2b portion of our ORION trial of AMX0035 in progressive supranuclear palsy, for which we have set a high bar and will inform our decision regarding advancing to the Phase 3 portion of the trial. We also look forward to providing an update on our Wolfram syndrome program later this year, building on previous long-term Week 48 data from our Phase 2 HELIOS trial. In ALS, we were pleased to receive FDA Fast Track designation for AMX0114, and we anticipate early cohort data from the ongoing Phase 1 LUMINA trial later this year. We remain focused on disciplined execution across our programs."

Second Quarter and Recent Updates:

- **Amylyx presented new exploratory analyses from the Phase 2 PREVENT and Phase 2b clinical trials of avexitide, a glucagon-like peptide-1 (GLP-1) receptor antagonist with U.S. Food and Drug Administration (FDA) Breakthrough Designation, for the treatment of post-bariatric hypoglycemia (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 GI 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 presented new long-term Week 48 data from the Phase 2 open-label HELIOS clinical trial of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO, also known as ursodoxicoltaurine]) in adults living with Wolfram syndrome at the Joint Congress of the European Society for Pediatric Endocrinology and the European Society of Endocrinology in May 2025.** Consistent with previously presented data on the primary efficacy outcome of improvement in pancreatic beta cell function, as measured by C-peptide response to a mixed-meal tolerance test at Week 24, treatment with AMX0035 through Week 48 of the HELIOS trial demonstrated continued and sustained improvement in pancreatic beta cell function. Long-term Week 48 results also showed sustained improvements or stabilization in multiple outcomes related to disease progression, including in glycemic control, as measured by hemoglobin A1c and time in target glucose range assessed by continuous glucose monitoring, as well as visual acuity. All participants with available measurements met the responder criteria, defined as either improvement or no change, on both the Patient Global Impression of Change and Clinician Global Impression of Change at Weeks 24 and 48, indicating stability or improvement in their Wolfram syndrome-related symptoms. Results from qualitative on-study interviews further supported the potential positive impact of AMX0035 on symptom burden. Safety data were consistent with prior studies of AMX0035.
- **Amylyx received FDA Fast Track designation for AMX0114, an investigational antisense oligonucleotide targeting knockdown of calpain-2, for people living with amyotrophic lateral sclerosis (ALS) in June 2025.** Under the FDA's Fast Track Designation, AMX0114 is eligible for more frequent meetings and communications with the FDA, as well as Priority Review if relevant criteria continue to be met.

Upcoming Expected Milestones:

- **Completion of recruitment for the pivotal Phase 3 LUCIDITY clinical trial of avexitide in PBH following RYGB**

surgery expected in 2025, with a data readout anticipated in the first half of 2026 and, if approved, commercial launch 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 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.

- **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. Phase 2b efficacy and safety data from an unblinded analysis with Week 24 data from all participants will be used to inform a go/no-go decision on the Phase 3 portion of the trial.
- **Update on the AMX0035 Wolfram syndrome program expected in 2025.** In 2024, Amylyx 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, including the trial's primary efficacy outcome of improvement in pancreatic function, as measured by C-peptide response to a mixed-meal tolerance test at Week 24. In May, long-term Week 48 data from HELIOS were presented at the Joint Congress of the European Society for Pediatric Endocrinology and the European Society of Endocrinology. These data demonstrated that treatment with AMX0035 led to continued sustained stabilization or improvement. These results and discussions with FDA are informing the design of a Phase 3 trial of AMX0035 in Wolfram syndrome.
- **Early cohort data from the Phase 1 LUMINA clinical trial of AMX0114 in ALS expected in 2025.** LUMINA is 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.

Financial Results for the Second Quarter Ended June 30, 2025

R&D Expenses: Research and development expenses for the second quarter of 2025 were $27.2 million, compared to $23.3 million for the same period in 2024. The increase was primarily due to the clinical development of avexitide in PBH and AMX0035 in PSP, offset by a decrease in spending on AMX0035 in ALS. Research and development expenses include $2.0 million of stock-based compensation expense for the quarter, compared to $2.4 million of stock-based compensation expense for the same period in 2024.

SG&A Expenses: Selling, general, and administrative expenses for the second quarter of 2025 were $15.6 million, compared to $21.6 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 $5.4 million of stock-based compensation expense for the quarter, compared to $7.1 million for the same period in 2024.

Net Loss: Net loss for the three months ended June 30, 2025 was $41.4 million, or $0.46 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 $180.8 million at June 30, 2025, compared to $204.1 million at March 31, 2025. Based on its current operating plans, Amylyx expects its cash runway to be through the end of 2026.

Investor Conference Call Information

Amylyx' management team will host a conference call today, August 7, 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 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 PSP or other neurodegenerative diseases and planned updates to those programs; 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, the expected timeline for data readout of the Phase 1 LUMINA clinical trial, and expectation for regulatory action; 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)

	June 30, 2025	December 31, 2024

Assets		
Cash, cash equivalents and marketable securities	$ 180,826	$ 176,501
Accounts receivable, net	103	447
Prepaid expenses and other current assets	5,961	12,484
Other assets	7,708	4,202
Total assets	$ 194,598	$ 193,634
Liabilities and Stockholders' Equity		
Accounts payable and accrued expenses	$ 20,759	$ 26,888
Other liabilities	5,962	1,981
Total liabilities	26,721	28,869
Stockholders' equity	167,877	164,765
Total liabilities and stockholders' equity	$ 194,598	$ 193,634

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,	
	2025	2024	2025	2024
Product revenue, net	$ —	$ (1,023)	$ —	$ 87,620
Operating expenses:				
Cost of sales	—	8	—	5,953
Cost of sales - inventory impairment and loss on firm purchase commitments	—	7,410	—	117,871
Research and development	27,217	23,347	49,336	59,955
Selling, general and administrative	15,640	21,647	31,324	79,406
Restructuring expenses	—	22,851	—	22,851
Total operating expenses	42,857	75,263	80,660	286,036
Loss from operations	(42,857)	(76,286)	(80,660)	(198,416)
Other income, net	1,414	3,586	3,310	7,165
Loss before income taxes	(41,443)	(72,700)	(77,350)	(191,251)
Provision for income taxes	—	—	—	242
Net loss	$ (41,443)	$ (72,700)	$ (77,350)	$ (191,493)
Net loss per share — basic and diluted	$ (0.46)	$ (1.07)	$ (0.88)	$ (2.82)
Weighted-average shares used in computing net loss per share — basic and diluted	89,138,568	68,024,929	87,427,345	67,939,642

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