Amylyx Pharmaceuticals Reports Fourth Quarter and Full Year 2024 Financial Results

March 4, 2025

- *Pivotal Phase 3 LUCIDITY trial of avexitide, a potential first-in-class GLP-1 receptor antagonist, in post-bariatric hypoglycemia underway; completion of recruitment expected in 2025, with topline data anticipated in first half of 2026*
- *Completed a public offering of common stock with net proceeds of approximately $65.5 million, extending the company's cash runway through the end of 2026*
- *Management to host conference call and webcast today at 8:00 a.m. Eastern Time*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 4, 2025-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial results for the fourth quarter and full year ended December 31, 2024.

"We made significant progress in 2024 across our pipeline, setting the stage for important milestones in the year ahead in orphan markets with high unmet need. We advanced our lead investigational asset avexitide into a pivotal Phase 3 trial and expect topline results in the first half of 2026. This year, we anticipate Week 48 data from our AMX0035 program in Wolfram syndrome, interim analysis data from our AMX0035 program in PSP, and early cohort data from our AMX0114 program in ALS," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "With a strong balance sheet, strengthened by our recent financing that extended our expected cash runway through the end of 2026, we believe we are well positioned to achieve our goals."

Fourth Quarter and Recent Updates:

- **Amylyx began the pivotal Phase 3 LUCIDITY clinical trial of avexitide, a glucagon-like peptide-1 (GLP-1) receptor antagonist, for the treatment of post-bariatric hypoglycemia (PBH).** In February 2025, the Company started recruiting for LUCIDITY, an approximately 75-participant, randomized, double-blind, placebo-controlled trial designed to evaluate efficacy and safety of avexitide in participants with PBH following Roux-en-Y gastric bypass (RYGB) surgery at approximately 20 sites in the U.S. Dosing is expected to begin in March or April of 2025. Amylyx presented the design of LUCIDITY in December 2024, and the poster is available on the "Publications" page of the Amylyx website. The initiation of LUCIDITY follows five PBH clinical trials of avexitide showing consistent, dose-dependent effects, including statistically significant reductions in hypoglycemic events and improved glucose control in PBH. Avexitide was generally well tolerated, with a favorable safety profile replicated across clinical trials. With these consistent data, Amylyx believes avexitide has the potential to become the first approved therapy for PBH.
- **Appointed Dan Monahan as Chief Commercial Officer to lead commercialization strategy.** Mr. Monahan joined Amylyx in January 2024 with over 20 years of experience launching industry-leading medicines at Otsuka, Novartis, and Sanofi. Mr. Monahan will lead the Company's commercialization efforts, including preparation for the potential launch of avexitide for the treatment of PBH.
- **Entered into a collaboration with Gubra A/S to develop a novel long-acting GLP-1 receptor antagonist.** Through this collaboration, Amylyx and Gubra anticipate identifying a lead development candidate to enter Investigational New Drug (IND)-enabling studies.
- **Amylyx began the Phase 1 LUMINA clinical trial of AMX0114, an investigational antisense oligonucleotide (ASO) targeting calpain-2, for people living with amyotrophic lateral sclerosis (ALS).** In February 2025, the Company started recruiting for LUMINA, a multicenter, randomized, placebo-controlled, multiple ascending dose 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. Dosing is expected to begin in March or April of 2025. 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.
- **Announced the closing of its underwritten public offering of 19.7 million shares of its common stock.** The net proceeds to Amylyx from this offering were approximately $65.5 million, extending the company's expected cash runway through the end of 2026.

Upcoming Expected Milestones:

- **Completion of enrollment for the pivotal Phase 3 LUCIDITY clinical trial of avexitide in PBH expected in 2025, with**

a data readout anticipated in the first half of 2026 and, if approved, commercial launch anticipated in 2027. LUCIDITY is evaluating the FDA-agreed-upon primary outcome of reducing hypoglycemic events through Week 16 and includes similar inclusion and exclusion criteria to previous Phase 2 trials of avexitide in PBH.

- **Amylyx plans to share Week 48 data from the ongoing Phase 2 HELIOS trial of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO, also known as ursodoxicoltaurine]) in Wolfram syndrome in the coming months.** Data from participants at Week 48 and regulatory interactions will inform the design of a Phase 3 trial of AMX0035 in Wolfram syndrome.
- **Unblinded interim analysis of the Phase 2b portion of the Phase 2b/3 ORION trial evaluating AMX0035 for progressive supranuclear palsy (PSP) 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 with a total of 139 participants randomized. Amylyx expects safety and efficacy data from an unblinded interim analysis in these participants through Week 24 and will use this data to inform a go/no-go decision on the Phase 3 portion of the trial.
- **Early cohort data from the Phase 1 LUMINA clinical trial of AMX0114 in ALS expected in 2025.** LUMINA clinical trial sites were activated in Canada in the beginning of 2025 and are recruiting participants. Amylyx is also working to open U.S. sites for screening, enrollment, and dosing.

Financial Results for the Fourth Quarter and Year Ended December 31, 2024

R&D Expenses: Research and development expenses for the fourth quarter of 2024 were $22.9 million, compared to $44.9 million for the same period in 2023, and $104.1 million for the year ended December 31, 2024, compared to $128.2 million for the year ended December 31, 2023. The decrease was primarily due to a decrease in spending on AMX0035 for the treatment of ALS, payroll and personnel-related costs, and a decrease in preclinical development activities. Research and development expenses include $1.8 million and $8.8 million of stock-based compensation expense for the quarter and year ended December 31, 2024, respectively compared to $2.8 million and $9.8 million of stock-based compensation expense for the quarter and year ended December 31, 2023, respectively.

SG&A Expenses: Selling, general, and administrative expenses for the fourth quarter of 2024 were $17.1 million, compared to $52.2 million for the same period in 2023 and $114.3 million for the year ended December 31, 2024, compared to $188.4 million for the year ended December 31, 2023. 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 $5.0 million and $24.3 million of stock-based compensation expense for the quarter and year ended December 31, 2024, respectively, compared to $6.8 million and $27.3 million of stock-based compensation expense for the quarter and year ended December 31, 2023, respectively.

Net Loss: Net loss for the three months ended December 31, 2024 was $37.5 million, or $0.55 per share, compared to net income of $4.7 million, or $0.07 per share on a fully diluted per share basis for the same period in 2023. Net loss for the year ended December 31, 2024 was $301.7 million, or $4.43 per share, compared with net income of $49.3 million, or $0.70 per share on a fully diluted per share basis for the year ended December 31, 2023.

Cash Position: Cash, cash equivalents, and short-term investments were $176.5 million at December 31, 2024, compared to $234.4 million at September 30, 2024. In addition, the company raised $65.5 million in January 2025. Based on its current operating plans, Amylyx expects its cash runway to be through the end of 2026. The cash position at December 31, 2024 reflects payments of $31.0 million in the fourth quarter of 2024 related to revenue returns and rebates, and the settlement of purchase commitments for AMX0035 that were established prior to the voluntary discontinuation of RELYVRIO®/ALBRIOZA™ inApril 2024. The remaining residual obligations related to the discontinuation of RELYVRIO/ALBRIOZA are $7.8 million, and the Company expects they will be paid through 2025.

Investor Conference Call Information
Amylyx' management team will host a conference call today, March 4, 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 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 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 affects approximately 8% of people who have undergone bariatric surgery, or 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 will be 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 will include a three-week run-in period and a 16-week double-blind treatment period. Participants who complete the double-blind period of the planned study 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; and expectations regarding the timing for recruitment completion and topline data readout of the Phase 3 avexitide trial in PBH; the potential of AMX0035 (sodium phenylbutyrate and taurursodiol) as a treatment for Wolfram syndrome and PSP or other neurodegenerative diseases; expectations regarding the timing of the announcement of Week 48 data from the Phase 2 HELIOS trial of AMX0035 for the treatment of Wolfram syndrome; planned discussions with regulatory authorities 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 and the expected timeline for data readout. 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)

	December 31,	
	2024	2023
Assets		
Cash, cash equivalents and marketable securities	$ 176,501	$ 371,362
Accounts receivable, net	447	40,050
Inventories	—	83,280
Prepaid expenses and other current assets	12,484	14,931
Other assets	4,202	7,831
Total assets	$ 193,634	$ 517,454
Liabilities and Stockholders' Equity		
Accounts payable and accrued expenses	$ 26,888	$ 79,785
Other liabilities	1,981	4,237
Total liabilities	28,869	84,022
Stockholders' equity	164,765	433,432
Total liabilities and stockholders' equity	$ 193,634	$ 517,454

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,	
	2024	2023	2024	2023
Product revenue, net	$ (665)	$ 108,449	$ 87,371	$ 380,786
Operating expenses:				
Cost of sales	—	9,360	5,953	25,441
Cost of sales - inventory impairment and loss on firm purchase commitments	—	—	118,680	—
Acquired in-process research and development	—	—	36,203	—
Research and development	22,892	44,914	104,084	128,187
Selling, general and administrative	17,097	52,241	114,331	188,356
Restructuring expenses	—	—	22,851	—
Total operating expenses	39,989	106,515	402,102	341,984
(Loss) income from operations	(40,654)	1,934	(314,731)	38,802
Other income, net	2,473	4,542	12,595	15,495
(Loss) income before income taxes	(38,181)	6,476	(302,136)	54,297
(Benefit) provision for income taxes	(635)	1,745	(393)	5,026
Net (loss) income	$ (37,546)	$ 4,731	$ (301,743)	$ 49,271
Net (loss) income per share				
Basic	$ (0.55)	$ 0.07	$ (4.43)	$ 0.73
Diluted	$ (0.55)	$ 0.07	$ (4.43)	$ 0.70
Weighted-average shares used in computing net (loss) income per share				
Basic	68,593,499	67,414,669	68,142,158	67,234,465
Diluted	68,593,499	69,196,421	68,142,158	69,991,340

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