Amylyx Pharmaceuticals Announces U.S. Expanded Access Program for Adults with Post-Bariatric Hypoglycemia

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- *The Expanded Access Program allows eligible adults with post-bariatric hypoglycemia following Roux-en-Y gastric bypass surgery access to avexitide, an investigational, first-in-class glucagon-like peptide-1 receptor antagonist*
- *Avexitide is being evaluated in the pivotal Phase 3 LUCIDITY clinical trial, with topline data readout anticipated in Q3 2026*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 5, 2026-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today announced the launch of a U.S. Expanded Access Program (EAP) for up to 250 adults with post-bariatric hypoglycemia (PBH) to provide treatment access to avexitide, an investigational, first-in-class glucagon-like peptide-1 (GLP-1) receptor antagonist.

The EAP allows U.S. physicians to request avexitide for adults with PBH following Roux-en-Y gastric bypass (RYGB) surgery who have a serious unmet medical need, are unable to participate in an ongoing clinical trial, have exhausted available management options, and meet all other eligibility criteria. 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.

"Listening to the PBH community is central to our work, and this dialogue directly informed our approach to the U.S. Expanded Access Program for avexitide," said Camille L. Bedrosian, MD, Chief Medical Officer at Amylyx. "The experiences shared by those living with PBH underscore the profound unmet medical need they face every day. This program reflects our commitment to providing a potential option for eligible individuals as we continue to advance avexitide through clinical development."

Avexitide is an investigational drug and has not been approved by the U.S. Food and Drug Administration (FDA) for any indication. Avexitide is being evaluated in the pivotal Phase 3 LUCIDITY clinical trial, a 16-week, multicenter, randomized, double-blind, placebo-controlled trial evaluating the efficacy and safety of avexitide in adults with PBH following RYGB surgery. Participants who complete the 16-week double-blind period are eligible to enter a 32-week open-label extension period. The trial has enrolled 78 participants, with topline data readout anticipated in Q3 2026. If approved, commercial launch of avexitide is anticipated in 2027.

Physician Inquiry and Patient Eligibility

Individuals with PBH who are interested in learning more about potential access to avexitide through the EAP should speak with their treating physician or care team to determine whether they may be eligible. Access to avexitide through the EAP is limited, may change over time, and participation is not guaranteed. Additional information about the EAP is available at amylyx.com/global-access-policy.

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 competitive GLP-1 receptor antagonist designed to 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; expectations regarding the timing for topline data readout of the Phase 3 LUCIDITY trial of avexitide; and expectations regarding timing for potential commercialization of avexitide. Any forward-looking statements in this press release 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; the risk that early-stage results may not reflect later-stage results; 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 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.

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