Amylyx Pharmaceuticals Announces Peer-Reviewed Publication of Phase 2 Open-Label HELIOS Trial Data for AMX0035 in The Journal of Clinical Investigation

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- *Peer-reviewed publication reports Week 24 and Week 48 results from Phase 2 HELIOS trial of AMX0035 in Wolfram syndrome, reinforcing 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*
- *The Company continues to work with the FDA on a Phase 3 trial in Wolfram syndrome*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 15, 2026-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today announced that Week 24 and Week 48 results from the Phase 2 open-label HELIOS clinical trial of AMX0035, an oral, fixed-dose combination of sodium phenylbutyrate and taurursodiol, in adults living with Wolfram syndrome have been published in *The Journal of Clinical Investigation*, a peer-reviewed medical journal.

The publication reports Week 24 and Week 48 results from the Phase 2 open-label HELIOS trial previously presented in part at medical meetings. Consistent with earlier disclosures, the findings indicate continued improvements observed in pancreatic beta cell function through Week 48, as measured by C-peptide response to a mixed-meal tolerance test. Additionally, secondary measures of glycemic control, including HbA1c and time in target glucose range (70–180 mg/dL), demonstrated improvement from baseline at Weeks 24 and 48, while best-corrected visual acuity trended toward stabilization over the 48-week period. Participant and Clinician Global Impression of Change assessments also classified participants with available data as responders, defined by improvement or disease stabilization.

"Wolfram syndrome is a rare genetic disease that presents significant challenges for patients and their families. It typically begins in childhood, with insulin-requiring diabetes and progressive optic nerve changes affecting vision, and over time can involve broader neurological symptoms that increasingly affect daily life, with no disease-modifying treatments approved today," said Fumihiko Urano, MD, PhD, Principal Investigator of the HELIOS trial and the Samuel E. Schechter Professor of Medicine in the Division of Endocrinology, Metabolism & Lipid Research at Washington University School of Medicine in St. Louis. "In this context, the publication of the HELIOS results at Weeks 24 and 48 is highly encouraging. The observed objective improvement in pancreatic function and visual acuity stabilization over 48 weeks, combined with a favorable safety profile, reinforces AMX0035's potential to meaningfully alter the trajectory of this disorder."

"We are excited to share the peer-reviewed publication of these results, which details the clinical findings of AMX0035 at Week 24 and 48 across both the endocrine and neurodegenerative manifestations of Wolfram syndrome. These findings are encouraging given that all trial participants were at least 17 years old, and their disease course had advanced considerably by the time of enrollment," said Camille L. Bedrosian, MD, Chief Medical Officer at Amylyx. "We extend our deepest gratitude to Dr. Urano, the clinical trial site staff, and most importantly, the trial participants and their families. Your unwavering dedication makes this research possible as we continue to work with the FDA on a Phase 3 clinical trial in Wolfram syndrome."

The peer-reviewed publication also details qualitative interviews that reinforced the clinical relevance of these objective outcomes, with participants indicating meaningful improvements in diabetes and vision problems. Furthermore, the published safety findings remained consistent with prior clinical experience with AMX0035, with reported adverse events being mild or moderate and no serious adverse events related to treatment.

HELIOS is a single-site, single-arm, open-label, Phase 2 clinical trial investigating AMX0035 in 12 adults living with Wolfram syndrome for up to 208 weeks followed by a four-week safety follow-up. The Company expects to present additional longer-term data from HELIOS at an upcoming scientific meeting.

About AMX0035
AMX0035 is an oral, fixed-dose combination of sodium phenylbutyrate (PB) and taurursodiol (TURSO; also known as ursodoxicoltaurine outside of the U.S.). AMX0035 was designed to slow or mitigate neurodegeneration by targeting endoplasmic reticulum (ER) stress and mitochondrial dysfunction, two connected central pathways that lead to cell death and neurodegeneration. Amylyx believes that its proprietary combination of PB and TURSO and their complementary mechanisms of action will allow us to synergistically target abnormal cell death to better prevent neurodegeneration than treatment targeted at either mechanism of action alone. The FDA and the European Commission granted Orphan Drug Designation to AMX0035 for the treatment of Wolfram syndrome in November 2020 and August 2024, respectively.

About Wolfram Syndrome

Wolfram syndrome is a rare, monogenic, progressive neurodegenerative disorder that progressively impacts multiple organs and systems. Wolfram syndrome is characterized by childhood-onset diabetes mellitus, optic nerve atrophy, and neurodegeneration. Common manifestations of Wolfram syndrome include diabetes mellitus and diabetes insipidus, gradual vision loss leading to blindness, hearing loss, neurogenic bladder, difficulties with balance and coordination, and difficulty breathing that can lead to respiratory failure and premature mortality. Wolfram syndrome is most commonly caused by pathogenic variants in Wolfram syndrome type 1 gene (*WFS1*). Because of the clear link between *WFS1* mutations and endoplasmic reticulum (ER) stress, Wolfram syndrome is considered a prototypical ER stress disorder. Wolfram syndrome affects approximately 3,000 people living in the U.S., and there are currently no FDA-approved treatment options.

About the HELIOS Trial
HELIOS (NCT05676034) is a 12-participant, single-site, single-arm, open-label, Phase 2 trial designed to evaluate the safety and tolerability of AMX0035, as well as its effects on various measures of endocrinological, neurological, and ophthalmologic function in adult participants living with Wolfram syndrome. Participants in HELIOS receive AMX0035 for up to 208 weeks followed by a four-week safety follow-up. Primary and secondary outcomes are assessed at Week 24 and at longer-term time points.

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 AMX0035 (sodium phenylbutyrate and taurursodiol) as a treatment for Wolfram syndrome; and plans for a Phase 3 trial of AMX0035 in Wolfram syndrome. 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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