Amylyx Pharmaceuticals Announces Completion of Enrollment in HELIOS, a Phase 2 Study of AMX0035 for the Treatment of Wolfram Syndrome

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Preliminary results from HELIOS anticipated in the second half of 2024

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 8, 2024-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) ("Amylyx" or the "Company") today announced it has completed enrollment of its Phase 2 HELIOS trial of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO]) for the treatment of Wolfram syndrome (WS), a rare genetic disease that typically results in neurodegeneration and premature death, which has no known cure. The trial has enrolled 12 adult participants living with WS, and preliminary results are anticipated in the second half of 2024.

"The unmet need for people living with WS remains significant. WS is progressive, often fatal, and difficult to diagnose, with current treatments focused mostly on helping to address symptoms," said Fumihiko Urano, MD, PhD, Principal Investigator of the HELIOS clinical trial and Professor of Medicine in the Division of Endocrinology, Metabolism & Lipid Research at Washington University School of Medicine. "Our preclinical findings showed that AMX0035 may impact relevant WS disease pathways and manifestations, and we look forward to the preliminary results from HELIOS anticipated later this year."

HELIOS is an open-label proof of biology trial designed to study the effect of AMX0035 on safety and tolerability, and various measures of endocrinological, neurological, and ophthalmologic function in adult participants living with WS. Data from this initial study will inform potential future Amylyx trials for individuals with WS.

More information on the HELIOS clinical study can be found at www.clinicaltrials.gov, NCT05676034.

In September 2022, researchers from Washington University School of Medicine in St. Louis, in collaboration with Amylyx, published preclinical data in the peer-reviewed *Journal of Clinical Investigation Insight*. The data explored the potential of AMX0035 as a novel therapeutic approach for WS and provided initial proof-of-concept for its therapeutic development in WS.

"Little progress has been made in the treatment of WS, and we are excited by the potential we have been seeing in the preclinical work with AMX0035," said Camille L. Bedrosian, MD, Chief Medical Officer of Amylyx. "These preclinical findings suggest the possibilities of AMX0035 for individuals with WS that we continue to explore, including in the HELIOS trial. We are grateful to those who partnered to complete this important clinical trial enrollment milestone, in particular, the WS community, their families and caregivers, experts, clinicians, researchers, and advocacy leaders."

Amylyx announced that the FDA granted orphan drug designation to AMX0035 for the treatment of WS in November 2020. The FDA may grant this designation to drugs and biologics intended to treat a rare disease or condition affecting fewer than 200,000 persons in the U.S. Orphan designation qualifies a company for certain benefits, including financial incentives to support clinical development and the potential for seven years of market exclusivity in the U.S. upon regulatory approval.

About Wolfram Syndrome

Wolfram syndrome (WS) is an autosomal recessive neurodegenerative disease characterized by childhood-onset diabetes, optic nerve atrophy, and neurodegeneration. Common manifestations of WS include diabetes mellitus, optic nerve atrophy, central diabetes insipidus, sensorineural deafness, neurogenic bladder, and progressive neurologic difficulties. Genetic and experimental evidence suggest that endoplasmic reticulum (ER) dysfunction is a critical pathogenic component of WS. The prognosis of WS is poor, and many people with the disease die prematurely with severe neurological disabilities.

About AMX0035 / RELYVRIO®/ ALBRIOZA™ / ALBRIOZA®

AMX0035 is an oral, fixed-dose combination of sodium phenylbutyrate and taurursodiol (known as ursodoxicoltaurine outside of the U.S.). It is approved as RELYVRIO® to treat amyotrophic lateral sclerosis (ALS) in adults in the U.S. and approved with conditions as ALBRIOZA™ for the treatment of ALS in Canada. AMX0035 is being studied for the potential treatment of other neurodegenerative diseases, and Amylyx is exploring its treatment in other populations and regions. The formulation of RELYVRIO, ALBRIOZA, and AMX0035 is identical.

About Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is committed to supporting and creating more moments for the neurodegenerative community through the discovery and development of innovative new treatments. Amylyx is headquartered in Cambridge, Massachusetts and has operations in Canada, EMEA, and Japan. For more information, visit amylyx.com and follow us on LinkedIn and X, formerly known as Twitter. 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, the potential of AMX0035 as a treatment for ALS and the Company's plans to explore the use of AMX0035 for other neurodegenerative diseases including Wolfram Syndrome, the timelines for the HELIOS study in WS, and expectations regarding our longer-term strategy. 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: Amylyx' ability to fund operations, the success, cost, and timing of Amylyx' program development activities, Amylyx' ability to execute on its commercial and regulatory strategy, regulatory developments, expectations regarding the timing and outcome of EMA's review of AMX0035 for the treatment of ALS, Amylyx' reliance on third parties, including to conduct clinical trials and manufacture products, and the effect of global economic uncertainty and financial market volatility caused by economic effects of rising inflation and interest rates, the COVID-19 pandemic, geopolitical instability, changes in international trade relationships and military conflicts, as well as the risks and uncertainties set forth in Amylyx' United States Securities and Exchange Commission (SEC) filings, including Amylyx' Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, 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. Subject to any obligations under applicable law, 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.

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