Amylyx Pharmaceuticals Announces Completion of Enrollment in Global Phase 3 PHOENIX Trial of AMX0035 in ALS

February 2, 2023

- Topline results expected in 2024

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 2, 2023-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) ("Amylyx" or the "Company") today announced the completion of enrollment in PHOENIX, a global, 48-week, randomized, placebo-controlled Phase 3 clinical trial of AMX0035 (sodium phenylbutyrate and taurursodiol [also known as ursodoxicoltaurine]) in people living with amyotrophic lateral sclerosis (ALS). Amylyx anticipates topline results in 2024. The study enrolled 664 participants living with ALS.

"The Phase 3 PHOENIX trial was an excellent collaboration between European and United States Centers for excellence in ALS research and care. We anticipate that PHOENIX will help us generate further data about the safety and efficacy of AMX0035," said Leonard H. van den Berg, MD, PhD, Professor of Neurology at UMC Utrecht in the Netherlands and Chairman of the Treatment Research Initiative to Cure ALS (TRICALS).

"We are pleased to share this milestone from the PHOENIX trial, a collaborative effort to advance our scientific understanding of ALS and build upon the positive data from our Phase 2 CENTAUR trial," added Sabrina Paganoni, MD, PhD, principal investigator of the CENTAUR study, investigator at the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital, and member of the Executive Committee of the Northeast Amyotrophic Lateral Sclerosis Consortium (NEALS).

The primary efficacy outcome of PHOENIX will be a joint assessment of Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) total score progression over 48 weeks, adjusted for mortality. Safety and tolerability will be assessed over 48 weeks.

Participants were randomized 3:2 to receive either AMX0035 or placebo for a 48-week period. PHOENIX spans more than 65 sites across Europe and the U.S., the majority of which are members of the NEALS or TRICALS consortia.

European participants completing the 48-week trial have the option to enroll in an open label extension (OLE) phase. During this phase, all participants receive AMX0035, and continued safety and efficacy measures will be assessed.

The design of PHOENIX was informed by the results of the Phase 2 CENTAUR clinical trial of AMX0035, which demonstrated a statistically significant benefit in function, as well as an observed benefit on survival in a longer-term post hoc analysis. Overall, reported rates of adverse events and discontinuations in CENTAUR were similar between AMX0035 and placebo groups during the 24-week randomized phase; however, gastrointestinal events occurred with greater frequency (≥2%) in the AMX0035 group.

"We are grateful for the people living with ALS and their families who are participating in PHOENIX and the dedication of the study investigators," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "We remain committed to continuing research and exploring the full potential of AMX0035 as part of our mission to one day end the suffering caused by ALS and other neurodegenerative diseases."

Marketing Application Reviews

The European Medicines Agency (EMA) is reviewing the submission of the Company's Marketing Authorisation Application for AMX0035 for the treatment of ALS in Europe. The Company anticipates a decision from the EMA in the first half of 2023. It is expected that the ongoing Phase 3 PHOENIX clinical trial will be part of the obligations of the conditional marketing authorization if granted.

In Canada, AMX0035 (marketed as ALBRIOZA™) was approved under Health Canada's Notice of Compliance with Conditions (NOC/c) policy. One of the conditions of the approval is the provision of data from the Phase 3 PHOENIX trial.

About AMX0035

AMX0035 is an oral, fixed-dose medication approved to treat amyotrophic lateral sclerosis (ALS) in adults in the U.S. as RELYVRIO™ (sodium phenylbutyrate and taurursodiol) and approved with conditions in Canada as ALBRIOZA™ (sodium phenylbutyrate and ursodoxicoltaurine). Additionally, the European Medicines Agency (EMA) is reviewing the Company's Marketing Authorisation Application for AMX0035 for the treatment of ALS in Europe. AMX0035 is being explored for the potential treatment of other neurodegenerative diseases.

About ALS

ALS is a relentlessly progressive and fatal neurodegenerative disorder caused by motor neuron death in the brain and spinal cord. Motor neuron loss in ALS leads to deteriorating muscle function, the inability to move and speak, respiratory paralysis and eventually, death. More than 90% of people with ALS have sporadic disease, showing no clear family history. ALS affects approximately 29,000 people in the U.S. and more than 30,000 people are estimated to be living with ALS in Europe (European Union and United Kingdom). People living with ALS have a median survival of approximately two years from diagnosis.

About Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is committed to supporting and creating more moments for the neurodegenerative disease community through the discovery and development of innovative new treatments. Amylyx is headquartered in Cambridge, Massachusetts and has operations in Canada and EMEA. For more information, visit amylyx.com and follow us on LinkedIn and 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, statements regarding the timing and results of the global Phase 3 PHOENIX trial; the potential approval of AMX0035 for the treatment of ALS in countries other than the United States and Canada; the potential of AMX0035 as a treatment for ALS and the Company’s plans to explore the use of AMX0035 for other neurodegenerative diseases; 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 of EMA review of AMX0035 for the treatment of ALS, Amylyx’ reliance on third parties, including to conduct clinical trials and manufacture products, and ongoing impacts of the COVID-19 pandemic on Amylyx’ operations, 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, 2022, 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.

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