Amylyx Pharmaceuticals Receives Negative CHMP Opinion on its Marketing Authorisation Application for AMX0035 for the Treatment of ALS in the European Union Following Re-Examination Process

October 13, 2023

- PHOENIX Phase 3 trial topline results on track for mid-2024 and will inform regulatory next steps in the EU
- Final decision from the European Commission expected by the end of 2023

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 13, 2023-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) ("Amylyx" or the "Company") today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) confirmed its initial negative opinion on the Marketing Authorisation Application (MAA) for AMX0035 (sodium phenylbutyrate and ursodoxicoltaurine [also known as taurursodiol]), under the trade name ALBRIOZA®, for the treatment of amyotrophic lateral sclerosis (ALS) in the European Union (EU). The decision follows the conclusion of the CHMP's formal re-examination procedure of an initial negative opinion adopted in June 2023.

ALBRIOZA was approved with conditions by Health Canada in June 2022 and granted a full approval by the U.S. Food and Drug Administration (FDA) under the trade name RELYVRIO® in September 2022.

The European Organization for Professionals and Patients with ALS (EUpALS) Patients and Carers Expert Board said in a statement, "We are disappointed to learn of this outcome, as it is a further setback for the more than 30,000 people living with ALS and their loved ones in Europe who have not seen therapeutic progress for this devastating disease in over 25 years."

"We share the frustration felt by the European ALS community, who has no time to wait for new, safe, and effective treatment options," said Stéphanie Hoffmann-Gendebien, General Manager and Head of EMEA at Amylyx. "We remain committed to exploring all potential paths forward in support of the Company's goal of getting AMX0035 to people living with ALS in the EU as quickly as possible."

"AMX0035 is the first and only drug to show an effect on both function and survival in the same trial. Since the medication's approval with conditions in Canada and full approval in the U.S., thousands of people have been prescribed AMX0035 in North America. ALS has no geographical boundaries, and we are working with urgency toward providing timely, broad, and sustainable access to AMX0035 for eligible people living with ALS who may benefit," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx.

Amylyx continues to focus on the completion of the PHOENIX Phase 3 clinical trial, which was initiated prior to its Marketing Authorisation Application submission and will provide additional data on the efficacy and safety profile of ALBRIOZA in people living with ALS. If PHOENIX is supportive, Amylyx plans to seek approval in the EU as quickly as possible. Topline results are anticipated in mid-2024.

PHOENIX is a 48-week, randomized placebo-controlled global Phase 3 clinical trial further evaluating the safety and efficacy of AMX0035 for the treatment of ALS. The study enrolled 664 participants living with ALS across 69 sites in either Europe or the U.S., the majority of which are members of the NEALS or TRICALS consortia. The design of PHOENIX was informed by the results of the Phase 2 CENTAUR clinical trial of AMX0035, which met its prespecified primary outcome and 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. More information on the PHOENIX trial can be found at https://classic.clinicaltrials.gov/ct2/show/NCT05021536 or https://www.clinicaltrialsregister.eu/ctr-search/search, EudraCT Number: 2021-000250-26.

The CHMP opinion will be forwarded to the European Commission, who will adopt the final decision on this application. A decision is anticipated by the end of 2023.

About RELYVRIO®/ ALBRIOZA™ / ALBRIOZA® / AMX0035

RELYVRIO®, an oral, fixed-dose combination of sodium phenylbutyrate and taurursodiol (known as ursodoxicoltaurine outside of the U.S.), is approved 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 are identical.

About the CENTAUR Trial

CENTAUR was a multicenter Phase 2 clinical trial in 137 participants with ALS encompassing a 6-month randomized placebo-controlled phase and an open-label extension (OLE) long-term follow-up phase. The trial met its primary efficacy endpoint.

Detailed safety and functional efficacy data from CENTAUR were published in the *New England Journal of Medicine*. Data from additional analyses from the CENTAUR trial were published in *Muscle & Nerve* in 2020 and 2022, and the *Journal of Neurology, Neurosurgery, and Psychiatry* in 2022.

The CENTAUR trial was funded, in part, by the ALS ACT grant and the ALS Ice Bucket Challenge, and was supported by The ALS Association, ALS Finding a Cure (a program of The Leandro P. Rizzuto Foundation), the Northeast ALS Consortium, and the Sean M. Healey & AMG Center for ALS at Mass General.

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.eu 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, 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 plans to seek approval in the future in Europe; 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 outcome of the re-examination of AMX0035 for the treatment of ALS in the EU, and 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 June 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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