Amylyx Pharmaceuticals Announces FDA Advisory Committee Supports Approval of AMX0035 for the Treatment of ALS

September 7, 2022

- FDA Advisory Committee voted 7:2 that the available evidence of effectiveness is sufficient to support approval of AMX0035 for the treatment of ALS

- If approved, AMX0035 will be the first treatment in ALS that has demonstrated a significant slowing of disease progression and functional decline, as well as extended survival, in a randomized, placebo-controlled clinical trial, as a standalone therapy or when added to existing approved treatments

- In December 2021, the FDA granted Priority Review and is expected to make a decision on AMX0035 by September 29, 2022, under the Prescription Drug User Fee Act

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 7, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") announced today that the U.S. Food and Drug Administration's (FDA) Peripheral and Central Nervous System Drugs Advisory Committee (PCNSDAC) voted (7 yes votes and 2 no votes) that the available evidence of effectiveness is sufficient to support approval of AMX0035 (sodium phenylbutyrate and taurursodiol [also known as ursodoxicoltaurine]) for the treatment of amyotrophic lateral sclerosis (ALS).

The PCNSDAC's decision was based on a review of all available evidence, including new analyses submitted for discussion at the September 7 meeting and the information presented at the March 30, 2022, PCNS meeting.

"The Committee's thoughtful review of the data and support of the benefit that AMX0035 may bring to the ALS community, if approved, is promising," said Jamie Timmons, M.D., Head of Scientific Communications of Amylyx. "The CENTAUR trial data has consistently demonstrated potential benefits of AMX0035 on function and overall survival. We are grateful to the advocacy community, our trial participants and their family members, the ALS clinicians, and countless others who continue to support our mission of ensuring that people living with ALS around the world can access promising new therapies as quickly and efficiently as possible."

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.

The PCNSDAC recommendations, while not binding, will be considered by the FDA in its review of the pending New Drug Application (NDA) for AMX0035. As previously reported, the Prescription Drug User Fee Act target action date for the NDA is September 29, 2022, which was extended by the FDA to allow more time to review additional analyses of data from the Company's clinical studies.

About AMX0035

AMX0035 (sodium phenylbutyrate and taurursodiol) is an oral fixed-dose medication with marketing applications pending in the United States and European Union and approved with conditions as ALBRIOZA™ to treat amyotrophic lateral sclerosis (ALS) in Canada. The combination of sodium phenylbutyrate and taurursodiol may reduce neuronal cell death, hypothesized to occur by simultaneously mitigating endoplasmic reticulum (ER) stress and mitochondrial dysfunction. AMX0035 is also being explored for the potential treatment of other neurodegenerative diseases.

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. Administration of AMX0035 (plus standard of care) significantly slowed the rate of functional decline as measured by the Amyotrophic Lateral Sclerosis Functional Rating Scale – Revised (ALSFRS-R) total score compared to placebo (plus standard of care) at the end of the 24-week randomized phase.

CENTAUR results appear to show that AMX0035 was generally well tolerated. Similar rates of adverse events and discontinuations were recorded in the AMX0035 and placebo groups during the 24-week randomized phase. However, gastrointestinal (GI) events occurred with greater frequency (≥2%) in the AMX0035 group. 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 the PHOENIX Trial

The Phase 3 PHOENIX clinical trial (NCT05021536) is a 48-week, randomized placebo-controlled global clinical trial further evaluating the safety and efficacy of AMX0035 (sodium phenylbutyrate and taurursodiol) for the treatment of ALS. The primary efficacy outcome of the trial will be a composite measure of Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) total score progression over 48 weeks and survival over 48 weeks. Secondary endpoints include change in slow vital capacity (SVC), measured both at home using a self-administered spirometer to support virtual data collection and at clinic sites using standard spirometry, quality of life patient-reported outcome assessments, ventilation-free survival rates and other measures. PHOENIX remains underway (recruitment has completed in the U.S. and remains ongoing in Europe) with initial results expected in 2024. More information on the PHOENIX trial can be found at clinicaltrials.gov and eudract.ema.europa.eu.

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 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 beliefs about the clinical study analyses of AMX0035, the potential additional regulatory approvals of AMX0035 and the Company’s efforts to advance such potential approvals and the potential of AMX0035 or other future therapeutic candidates as a treatment for ALS, 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: the success, cost, and timing of Amylyx’ program development activities, Amylyx’ ability to execute on its strategy, regulatory developments, expectations regarding the timing of regulatory review of AMX0035 and the inherent unpredictability of the regulatory review process, Amylyx’ ability to fund operations, and the impact that the ongoing COVID-19 pandemic will have on Amylyx’ operations, as well as those risks and uncertainties set forth in Amylyx’ United States Securities and Exchange Commission (SEC) filings, including Amylyx’ Annual Report on Form 10-K for the year ended December 31, 2021, 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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