Amylyx Pharmaceuticals Announces Outcome of FDA Advisory Committee Meeting on AMX0035 for the Treatment of ALS

March 30, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 30, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") announced today the outcome of the U.S. Food and Drug Administration's (FDA) Peripheral and Central Nervous System Drugs Advisory Committee (PCNSDAC) meeting to review the Company's New Drug Application (NDA) for AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO; also known as ursodoxicoltaurine]) for the treatment of amyotrophic lateral sclerosis (ALS).

On the question, "Do the data from the single randomized, controlled trial and the open-label extension study [Phase 2 CENTAUR trial] establish a conclusion that sodium phenylbutyrate/taurursodiol [AMX0035] is effective in the treatment of patients with amyotrophic lateral sclerosis (ALS)?," the PCNSDAC voted 4 (yes) and 6 (no).

"We remain confident in the data from the Phase 2 CENTAUR trial and the potential benefits of AMX0035 as a treatment option for people living with ALS," said Jamie Timmons, M.D., Head of Scientific Communications of Amylyx. "We are motivated by the hundreds of people impacted by this devastating disease who shared their personal testimonies, both written and spoken, in conjunction with today's meeting. We are also encouraged by the expert ALS physicians who shared their clinical perspectives. Our application is under review by the FDA, and we remain committed to pursuing its approval given the pressing need for new treatments for ALS."

Although the FDA considers the recommendations of its advisory committees, the recommendations by the panel are non-binding. The final decision regarding approval of a pending NDA is made by the FDA. As previously reported, the FDA has granted Priority Review to the NDA for AMX0035 and is expected to make a decision on the approval of AMX0035 by June 29, 2022 under the Prescription Drug User Fee Act.

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.

About AMX0035

AMX0035 is a proprietary oral fixed-dose combination of two small molecules: sodium phenylbutyrate (PB), which is a small molecular chaperone designed to reduce the unfolded protein response (UPR), preventing cell death resulting from the UPR, and taurursodiol (TURSO; also known as ursodoxicoltaurine), which is a Bax inhibitor designed to reduce cell death through apoptosis. PB and TURSO were combined in a fixed-dose formulation in an effort to reduce neuronal death and dysfunction. AMX0035 is designed to target the endoplasmic reticulum and mitochondrial-dependent neuronal degeneration pathways in ALS and 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 long-term follow-up phase. The trial met its primary efficacy endpoint of reducing functional decline as measured by the ALS Functional Rating Scale-Revised (ALSFRS-R).

Overall, reported rates of adverse events and discontinuations 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. Detailed data from CENTAUR is published in the *New England Journal of Medicine* (NEJM) and *Muscle and Nerve*.

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 Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company working on developing a novel therapeutic for ALS and other neurodegenerative diseases. For more information, visit www.amylyx.com and follow us on LinkedIn and Twitter. For investors please visit www.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 the of 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 potential regulatory approval of AMX0035 and the potential of AMX0035 as a treatment for ALS; and expectations regarding our longer-term strategy. Any forward-looking statements in this statement 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, 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 filings. 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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