Amylyx Pharmaceuticals Announces Launch of U.S. Expanded Access Program for AMX0035

March 18, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 18, 2022-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) ("Amylyx" or the "Company") announced today the launch of an Expanded Access Program (EAP) for AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO; also known as ursodoxicoltaurine]) in the U.S. for people living with amyotrophic lateral sclerosis (ALS) that meet program eligibility criteria.

"We take our responsibilities as part of the ALS community seriously, and we know access to therapies is of the utmost importance," said Machelle Manuel, Ph.D., Head, Global Medical Affairs. "In designing the EAP, we worked closely with the ALS community – people living with ALS, caregivers, advocacy groups, and clinicians – to take their needs and ideas into account."

EAPs are designed to give access to potential therapies before they are approved by the U.S. Food and Drug Administration (FDA) and may include people not typically eligible for clinical trials. The U.S. EAP for AMX0035 will be available to people with ALS meeting program eligibility requirements. More information, including available EAP program sites, can be found at www.clinicaltrials.gov, study A35-006, NCT05286372. The U.S. EAP will run in parallel with the ongoing Phase 3 PHOENIX trial (study A35-004, NCT05021536) and the FDA marketing application review. People with ALS who are eligible for PHOENIX will not be eligible for the U.S. EAP as the criteria for entry do not overlap.

Healthcare professionals interested in making a request for access to the EAP or people interested in learning more about the criteria for the program can visit www.clinicaltrials.gov, study A35-006, and/or email our Clinical Research Organization overseeing the program at patient.access@bionical-emas.com.

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 Expanded Access to AMX0035

Access to AMX0035 outside of clinical trials is available in the U.S. for certain adults with ALS who meet eligibility criteria for participation in the FDA-authorized Expanded Access Program (EAP) for AMX0035. To learn more about the currently open AMX0035 EAP, visit www.clinicaltrials.gov. At this time, access to AMX0035 for other uses, including right to try or individual patient access, is not available outside of the EAP. Additionally, access to AMX0035 outside the U.S. is currently not available outside of current clinical programs. To request more information about the U.S. EAP, contact patient.access@bionical-emas.com. For more information about other expanded access or current clinical trials, submit a request for information to medinfo@amylyx.com. The Company aims to acknowledge requests for information within 5 business days.

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 (PB/TURSO) for the treatment of ALS. The primary efficacy outcome of the trial will be a composite measure of survival and Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) total score progression over 48 weeks and survival and tolerability 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. More information on the PHOENIX trial can be found at www.clinicaltrials.gov and eudract.ema.europa.eu.

About Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company working on developing a novel therapeutic for amyotrophic lateral sclerosis (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 or other future therapeutic candidates as a treatment for neurodegenerative diseases. 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 its registration statement on Form S-1 filed with the United States Securities and Exchange Commission. 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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Source: Amylyx Pharmaceuticals, Inc.