Amylyx Pharmaceuticals Announces FDA Advisory Committee Meeting to Review New Drug Application for AMX0035 for the Treatment of ALS Scheduled for March 30, 2022

February 16, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) today announced that a virtual meeting of the U.S. Food and Drug Administration's (FDA) Peripheral and Central Nervous System Drugs Advisory Committee to review the New Drug Application (NDA) for AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO; also known as ursodoxicoltaurine]) has been scheduled for March 30, 2022. AMX0035 is an investigational therapy for the treatment of amyotrophic lateral sclerosis (ALS). As previously reported, the U.S. FDA has granted Priority Review and assigned a Prescription Drug User Fee Act date for AMX0035 of June 29, 2022.

"There are few treatments approved for ALS, a devastating disease that impacts a person's ability to move, speak, eat and breathe," said Lahar Mehta, M.D., Head of Global Clinical Development of Amylyx. "We look forward to a robust scientific discussion with the members of the advisory committee panel regarding the clinical data submitted to support our New Drug Application for AMX0035."

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 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 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 potential approval of AMX0035 for the treatment of ALS; 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 FDA review of AMX0035 for the treatment of ALS, 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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