Amylyx Pharmaceuticals Announces FDA Advisory Committee Will Reconvene to Review New Drug Application for AMX0035 for the Treatment of ALS on September 7, 2022

August 3, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 3, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) today announced that the U.S. Food and Drug Administration's (FDA) Peripheral and Central Nervous System Drugs Advisory Committee (PCNSDAC) will reconvene to review the New Drug Application (NDA) for AMX0035 (sodium phenylbutyrate and taurursodiol [also known as ursodoxicoltaurine]) for the treatment of amyotrophic lateral sclerosis (ALS). The second virtual meeting of the PCNSDAC to discuss the AMX0035 NDA is scheduled, as published in the Federal Register, for September 7, 2022.

The PCNSDAC previously met on March 30, 2022, to discuss the NDA for AMX0035 for the treatment of ALS. The FDA is reconvening the committee to discuss the additional analyses of data from the Company's clinical studies that were determined by the FDA to constitute a major amendment to the NDA. As a result, FDA extended the Prescription Drug User Fee Act (PDUFA) target action date for the AMX0035 NDA to September 29, 2022, from the original date of June 29, 2022.

"We are determined to do everything we can to bring AMX0035 to people living with ALS, and we look forward to another robust scientific discussion with the members of the advisory panel regarding the additional analyses from our clinical studies and the potential of AMX0035," said Jamie Timmons, M.D., Head of Scientific Communications of Amylyx. "We remain confident in the data, including the recently published analyses supporting the previously reported functional and overall survival benefit for AMX0035. There is an urgent unmet need for new and effective treatments in ALS, and our team is working nonstop to continue our mission to end the suffering caused by neurodegenerative diseases."

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 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 regulatory approval of AMX0035 and the Company's efforts to advance such potential approval and the potential of AMX0035 or other future therapeutic candidates as a treatment for ALS and other 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 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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