



Amylyx Pharmaceuticals Announces FDA Plan to Reconvene Advisory Committee to Review AMX0035 NDA for the Treatment of ALS on September 7, 2022

July 5, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 5, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today announced that the U.S. Food and Drug Administration ("FDA" or the "Agency") has informed the Company that the Agency is planning to reconvene the Peripheral and Central Nervous System Drugs Advisory Committee (PCNSDAC) to discuss the 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 Wednesday, September 7, 2022. The FDA will formally announce the scheduling of the planned PCNSDAC meeting in the Federal Register. Discussions will focus on 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. The Prescription Drug User Fee Act (PDUFA) target action date for the AMX0035 NDA is September 29, 2022, which was extended from June 29, 2022, to allow more time for the FDA to review additional analyses of data from the Company's clinical studies. The PCNSDAC previously met on March 30, 2022, to discuss the NDA for AMX0035 for the treatment of ALS.

"We remain engaged with the FDA to advance AMX0035 through the review process as efficiently as possible," said Tammy Sarnelli, Global Head of Regulatory Affairs of Amylyx. "We are pleased that the members of the advisory panel will review additional analyses from our clinical studies, including recently published analyses, supporting the previously reported functional and overall survival benefit for AMX0035. As we have heard from the ALS community, there is a crucial need for new and effective treatments in ALS, and our team will continue to work around the clock to advance treatments for ALS in the U.S."

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](https://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 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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