Amylyx Pharmaceuticals Announces Publication of Preclinical Data Showing Potential Synergistic Effect of AMX0035 Compared to Individual Compounds

September 12, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 12, 2022-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) ("Amylyx" or the "Company") today announced the publication of preclinical data showing the effect of sodium phenylbutyrate (PB) and taurursodiol (TURSO, also known as ursodoxicoltaurine) on the transcriptomic and metabolomic profiles of primary skin fibroblasts from adults with sporadic amyotrophic lateral sclerosis (ALS) and adults without ALS. These results, which showed that AMX0035 had a greater and distinct effect on genes and metabolites involved in ALS-relevant pathways as compared to either PB or TURSO alone, are published in the peer-reviewed medical journal, *Annals of Clinical and Translational Neurology*.

"ALS is a relentless and complex disease, and while AMX0035 has been shown to meaningfully slow the loss of function and extend survival in people living with ALS in a randomized, placebo-controlled clinical trial, this study is the first to explore the molecular effects of the combination of AMX0035 versus the individual compounds in ALS patient-derived cells," said Dr. Hibiki Kawamata and Dr. Giovanni Manfredi, leading authors of the study from the Feil Family Brain & Mind Research Institute, Weill Cornell Medicine. "We are pleased to demonstrate that the combination has a greater and distinct impact as compared to the individual compounds on primary skin fibroblasts from people with sporadic ALS, which affects more than 90% of people living with ALS. These findings may underlie the benefits that AMX0035 has been shown to have on individuals living with ALS."

Data analyses from twelve primary fibroblast lines from healthy donors and twelve lines derived from people with ALS demonstrated that treatment with AMX0035 changed more genes and metabolites than treatment with either PB or TURSO individually. The majority of changes in genes and metabolites were unique to treatment with AMX0035 versus PB or TURSO alone, suggesting that the combination of PB and TURSO has certain distinct effects that are not exclusively additive effects of PB and TURSO. Further, most changes affected the expression of genes involved in ALS-relevant pathways, including nucleocytoplasmic transport, unfolded protein response, mitochondrial function, RNA metabolism, and innate immunity. These studies add new learnings regarding the mechanistic activity of PB and TURSO and supplement our preclinical work that has shown synergistic reduction in neuronal death through the hypothesized mechanism of combined inhibition of endoplasmic reticulum and mitochondrial stress.

About AMX0035

AMX0035 (sodium phenylbutyrate and taurursodiol) is an oral fixed-dose medication approved with conditions as ALBRIOZA™ to treat amyotrophic lateral sclerosis (ALS) in Canada and with marketing applications pending in the United States and the European Union. 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 preclinical and clinical study analyses of AMX0035, the potential benefits of AMX0035 on individuals living with ALS, the Company's efforts to advance potential regulatory approval of AMX0035 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 press release 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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