Amylyx Pharmaceuticals to Announce Plans for an Open Label Extension (OLE) Phase for Global PHOENIX Trial of AMX0035 in ALS at the 17th International Congress on Neuromuscular Diseases

July 6, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jul. 6, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today announced a poster on the ongoing international Phase 3 PHOENIX trial (study A35-004, NCT05021536) of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO; also known as ursodoxicoltaurine]) in people living with amyotrophic lateral sclerosis (ALS) will be presented on Saturday, July 9, at the 17th International Congress on Neuromuscular Diseases (ICNMD 2022) in Brussels, Belgium. The poster will include an overview of the PHOENIX trial design and methodology, as well as an update on a planned open-label extension (OLE) phase for PHOENIX.

"The Phase 3 PHOENIX trial is enrolling a larger and broader group of people living with ALS than were enrolled in the Phase 2 CENTAUR trial, helping us to generate additional data on potential treatment effects of AMX0035," said Leonard H. van den Berg, M.D., Ph.D., Professor of Neurology at UMC Utrecht in the Netherlands and Chairman of the Treatment Research Initiative to Cure ALS (TRICALS), a large European trial network dedicated to finding effective treatments for ALS. "We look forward to sharing our plans for the OLE phase at ICNMD."

Presentation Details:

Title: International Phase 3 Trial Evaluating Sodium Phenylbutyrate and Taurursodiol in Amyotrophic Lateral Sclerosis (PHOENIX)
Poster Number: 11
Date: Saturday, July 9, 2022
Time: 12:45-2:15 p.m. CEST

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 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 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 (sodium phenylbutyrate and taurursodiol) 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 the CENTAUR Trial

CENTAUR was a multicenter Phase 2 clinical trial in 137 participants with ALS encompassing a 6-month randomized placebo-controlled phase and an open-label long-term follow-up phase. The trial met its primary efficacy endpoint of reducing functional decline as measured by the Amyotrophic Lateral Sclerosis Functional Rating Scale – Revised (ALSFRS-R).

Overall, reported rates of adverse events and discontinuations were similar between AMX0035 and placebo groups during the 24-week randomized phase; however, gastrointestinal events occurred with greater frequency (≥2%) in the AMX0035 group. Detailed safety and functional efficacy data from CENTAUR were published in the *New England Journal of Medicine*. Data from additional analyses from the CENTAUR trial were published in *Muscle & Nerve* in 2020 and 2022, and the *Journal of Neurology, Neurosurgery and Psychiatry* in 2022.

The CENTAUR trial was funded, in part, by the ALS ACT grant and the ALS Ice Bucket Challenge, and was supported by The ALS Association, ALS Finding a Cure (a program of The Leandro P. Rizzuto Foundation), the Northeast ALS Consortium, and the Sean M. Healey & AMG Center for ALS at Mass General.

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 the 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 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 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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