



## Amylyx Pharmaceuticals Announces Presentation of New Analysis of CENTAUR Trial Results at the European Network to Cure ALS 2022 Meeting

May 26, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 26, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") announced today that a new post hoc analysis measuring substantial individual response to AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO; also known as ursodoxicoltaurine]) will be presented at the European Network to Cure ALS (ENCALS) 2022 Meeting on June 2, 2022 in Edinburgh, Scotland.

"Determining whether an individual will have a substantial response to therapy is a challenge for the ALS field since ALS progresses at variable rates," said Lahar Mehta, M.D., Head of Global Clinical Development at Amylyx Pharmaceuticals and study co-author. "Given the heterogeneous nature of ALS, we developed a method in partnership with the scientific community to further clarify which participants had a substantial individual response to therapy and found a greater portion in the AMX0035 arm compared to placebo. These findings continue to demonstrate the efficacy of AMX0035 in people living with ALS and may provide a new methodological tool for the ALS field."

### Presentation Details:

#### Poster Presentation

**Title:** Determining Individual Substantial Response in Amyotrophic Lateral Sclerosis: Utilizing a New Method on CENTAUR Trial Results

**Poster Number:** Poster # i185

**Date:** Thursday, June 2, 2022

**Time:** 5:00 p.m. – 6:30 p.m. BST

### 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 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 ALS 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 ( $\geq 2\%$ ) 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 a clinical-stage biopharmaceutical company working on developing a novel therapeutic for amyotrophic lateral sclerosis (ALS) and other neurodegenerative diseases. For more information, visit [amylyx.com](https://amylyx.com) and follow us on [LinkedIn](#) and [Twitter](#). For investors, please visit [investors.amylyx.com](https://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 of AMX0035 as a treatment for ALS [and the ability of an individual response method to enable greater personalization for ALS treatments]; the potential of AMX0035 or other future therapeutic candidates as a treatment for neurodegenerative diseases generally; and expectations regarding our longer-term strategy. 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 commercial and regulatory 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 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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