Amylyx Pharmaceuticals Announces EMA Validation of Marketing Authorisation Application (MAA) for AMX0035 for the Treatment of ALS

February 25, 2022

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 25, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) (“Amylyx” or the “Company”), today announced that its Marketing Authorisation Application (MAA) to the European Medicines Agency’s (EMA) Committee for Medicinal Products for Human Use (CHMP) for AMX0035 (sodium phenylbutyrate [PB] and ursodoxicoltaurine [TURSO; also known as taurursodiol]) for the treatment of amyotrophic lateral sclerosis (ALS) has been validated and is now under CHMP review.

“The validation of our MAA for review by CHMP is another important step toward our goal of bringing a potential treatment option to people with ALS in Europe,” said Stéphanie Hoffmann-Gendebien, MBA, Head, General Manager in Europe, the Middle East and Africa (EMEA) of Amylyx. “We are building out our European team to be prepared to launch AMX0035 should the CHMP review result in a positive opinion, as we know people with ALS and their families have no time to wait.”

“Thank you to the large team of people who have made this milestone possible,” said Tammy Sarnelli, Global Head of Regulatory Affairs of Amylyx. “We look forward to continuing to work closely with EMA through the MAA review process.”

The MAA submission is based on data from the CENTAUR trial, a randomized, double-blind, placebo-controlled Phase 2 clinical trial conducted at 25 centers of the Northeast ALS Consortium (NEALS), evaluating 137 adults with ALS. In this trial, participants receiving AMX0035 demonstrated a statistically significant reduction in clinical decline at the end of the 6-month randomized phase as measured by the Revised ALS Functional Rating Scale (ALSFRS-R), the most widely used scale in clinical practice and in ALS clinical trials.

In a survival analysis conducted in all randomized participants from the CENTAUR trial who were followed for up to three years, which included participants who continued to receive AMX0035 in an open-label extension phase during the follow-up period, participants who started on AMX0035 during the placebo-controlled phase of CENTAUR demonstrated a 44% lower risk of death compared to those who started on placebo during the placebo-controlled phase (HR 0.56; 95% CI, 0.34-0.92). Median survival duration through the open-label long-term follow-up phase was 25.0 months (95% CI, 19.0-33.6 months) in the group that started on AMX0035 and 18.5 months (95% CI, 13.5-23.2 months) in the group that started on placebo, a 6.5-month difference.

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 data from CENTAUR is published in the *New England Journal of Medicine* (NEJM) and *Muscle and Nerve.*

“After many years and trial failures in ALS, AMX0035 has given us the hope that a new potential treatment option may be on the horizon for the approximately 40,000 people living with this disease in Europe,” said Evy Reviers, Chairwoman of European Organization for Professionals and Patients with ALS (EUpALS) and CEO of ALS Liga Belgium. “We are encouraged by this validation and look forward to seeing AMX0035 potentially progress through the European regulatory review process.”

“ALS is a devastating progressive disease that heavily impacts people living with the disease and their loved ones, and we are in urgent need of a new therapy. We highly encourage the mission of Amylyx to find an innovative treatment for ALS,” 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 a treatment for ALS.

Marketing Application Reviews and Phase 3 Clinical Trial

Amylyx currently has marketing applications under review for AMX0035 for the treatment of ALS in Canada and the United States. The U.S. FDA has granted Priority Review and assigned a Prescription Drug User Fee Act date for AMX0035 of June 29, 2022, the target date by which the FDA intends to complete its review and take action on the New Drug Application (NDA).

Enrollment for the global Phase 3 PHOENIX clinical trial (NCT05021536) is underway in the United States and Europe. The trial is designed to provide additional safety and efficacy data for AMX0035 for the treatment of ALS to further support Amylyx’ global regulatory efforts.

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 clinical 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, GI events occurred with greater frequency (≥2%) in the AMX0035 group.

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 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 (PB/TURSO) 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 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 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 www.amylyx.com and follow us on LinkedIn and Twitter. For investors please visit www.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 the of 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 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 its registration statement on Form S-1 filed with the United States Securities and Exchange Commission. 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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