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AMYLYX PHARMACEUTICALS PROVIDES GLOBAL REGULATORY UPDATE ON AMX0035 FOR ALS

– Marketing Authorization Application (MAA) Submission in Europe Planned for Q4 2021

– Phase 3 Trial in Europe and U.S. Expected to Begin in Q3 2021 to Support Regulatory Submissions

CAMBRIDGE, Mass.–(BUSINESS WIRE)–
Amylyx Pharmaceuticals, Inc., a pharmaceutical company focused on developing new treatments for amyotrophic lateral sclerosis (ALS), Alzheimer's Disease and other neurodegenerative diseases, today provided an update on its plans to advance AMX0035 through the clinical development process for the treatment of ALS. The company intends to submit a Marketing Authorization Application (MAA) for AMX0035 to the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) by the end of 2021. As previously reported, the company plans to submit a marketing application with Health Canada Q2 2021.

The FDA has expressed an interest in seeing data from an additional placebo-controlled clinical trial prior to receiving a New Drug Application (NDA), the vehicle through which a pharmaceutical sponsor formally proposes that the FDA approve an investigational product. To fulfill the FDA's request and to continue to build upon the growing body of evidence supporting AMX0035 for the potential treatment of ALS, Amylyx plans to initiate a Phase 3 clinical trial in Europe and the United States. The trial is expected to begin enrollment in Q3 2021. The FDA also expressed that it would continue to discuss with Amylyx how regulatory requirements may be met in the most expeditious way possible.

Amylyx is also continuing to discuss AMX0035 with additional regulatory bodies worldwide to determine the most appropriate path forward.

“People living with ALS need clinical answers and solutions quickly. We have been in close contact with physicians and global health authorities to rapidly evaluate AMX0035 for the treatment of ALS,” said Joshua Cohen, Co-CEO, Chairman and Co-Founder of Amylyx. “We are thrilled to plan our submissions in Europe and Canada and will continue working closely with regulators and the ALS community worldwide to determine the most expeditious and responsible pathways to advance AMX0035 through the clinical development process. We appreciate all of the advice and guidance from the regulators worldwide and will continue to act with haste and to keep the community updated.”

CENTAUR was a 24-week placebo-controlled study of 137 participants with ALS that evaluated the safety and efficacy of AMX0035. The trial was led by investigators at the Healey & AMG Center for ALS at Massachusetts General Hospital in collaboration with the Northeast ALS Consortium. As published in the *New England Journal of Medicine (NEJM)*, the study met its primary efficacy endpoint of slowing ALS progression as measured by the ALS Functional Rating Scale-Revised (ALSFRS-R). The rates of adverse events were similar between the AMX0035 and placebo arms of the study. However, discontinuations related to adverse events occurred more frequently in the AMX0035 arm than in the placebo arm.

Participants who completed CENTAUR were eligible to enroll in an open-label extension (OLE) and receive AMX0035. In a nearly three-year overall survival analysis of all randomized participants from CENTAUR that was published in *Muscle & Nerve,* those originally randomized to AMX0035 had a 44% lower risk of death compared to those originally randomized to placebo.

“With the results from CENTAUR, we showed that AMX0035 may provide people living with ALS hope and the chance to function better and live longer lives,” said Sabrina Paganoni, M.D., Ph.D., principal investigator of the CENTAUR trial, investigator at the Healey & AMG Center for ALS at Massachusetts General Hospital and Assistant Professor of PM&R at Harvard Medical School and Spaulding Rehabilitation Hospital. “We are very excited to see AMX0035 advancing on multiple regulatory fronts and remain optimistic that it can potentially help people living with ALS around the world.”

“ALS is a devastating progressive disease that impacts patients and their families not only physically, but also mentally and emotionally,” 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. “After decades of ALS trial

failures, AMX0035 has given us the hope that a new potential treatment option may be on the horizon for those living with ALS. We remain highly encouraged as AMX0035 continues to move through the regulatory review process, and are excited for the Phase 3 clinical trial."

"The pivotal Phase 3 clinical trial will catalyze a global collaboration between Amylyx, European and US ALS experts, advocacy groups, and clinical trial networks," said Merit Cudkowicz, MD, co-principal investigator of the CENTAUR trial and co-founder of the Northeast ALS Consortium, Director of the Healey & AMG Center for ALS and Chair of Neurology at Massachusetts General Hospital and the Julieanne Dorn Professor of Neurology at Harvard Medical School. "We hope this is just the beginning of providing new options to people living with ALS."

"People with ALS are fighting against this disease every day," said Evy Reviers, Chairwoman of European Organization for Professionals and Patients with ALS (EUpALS) and CEO of ALS Liga Belgium. "As a caregiver of a loved one with ALS, I know how critical it is to make potential treatment options available to the ALS community. On behalf of EUpALS, I look forward to working with industry, academia and health authorities to make that a reality."

"We are pleased to share our plans to initiate regulatory filing in Europe," said Justin Klee, Co-CEO and Co-Founder of Amylyx. "Every single day matters for people living with ALS and we will continue to work with the U.S. FDA and global regulatory agencies to meet their requests so that we can advance AMX0035 through the clinical development process as quickly as possible. We will share updates on our progress with the community as we have them."

About Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is a pharmaceutical company working on developing a novel therapeutic for amyotrophic lateral sclerosis (ALS), Alzheimer's disease and other neurodegenerative diseases. For more information, visit www.amylyx.com and follow us on LinkedIn and Twitter.

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