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Amylyx Pharmaceuticals Announces Plan to Submit New Drug Application (NDA) for AMX0035 for the Treatment of ALS in the Coming Months

Amylyx Pharmaceuticals announced its intention to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for AMX0035 (sodium phenylbutyrate (PB) and taurursodiol (TURSO)) for the treatment of amyotrophic lateral sclerosis (ALS). The decision by the company to submit the application in the coming months follows recent discussions with the FDA, including a pre-NDA meeting held on July 15, 2021.

“We are thrilled to move toward the U.S. submission of an NDA for AMX0035 and look forward to continuing to work with the FDA,” said Joshua Cohen, Co-CEO, Chairman and Co-Founder of Amylyx. “For those living with ALS, time is the most important resource, and we remain focused on advancing AMX0035 through the clinical development process as efficiently as possible,” added Justin Klee, Co-CEO, Director and Co-Founder of Amylyx. “We’re endlessly grateful for all of the support and efforts of ALS Finding a Cure, the ALS Association, I AM ALS, the Healey & AMG Center at Mass General and the Northeast ALS Consortium, and all CENTAUR trial participants for their critical involvement as we approach this milestone.”

“AMX0035 has shown potential to provide those living with ALS and their families hope for the future,” 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 learn of this positive development and optimistic that AMX0035 may make a real difference in the lives of people with ALS around the world.”

“This is another great step forward for Americans living with ALS. In the CENTAUR trial, led by the Northeast ALS Consortium and the Healey & AMG Center at Mass General, AMX0035 was found to both slow ALS progression and extend life,” said Merit Cudkowicz, M.D., 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.

As previously reported, Amylyx filed a New Drug Submission (NDS) for AMX0035 for the treatment of ALS with Health Canada in June 2021. Amylyx also 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 and initiate a global Phase 3 clinical trial with sites in Europe and the United States in the third quarter of 2021.

The Phase 3 PHOENIX trial (A35-004 PHOENIX) of AMX0035 for the treatment of people with ALS will assess the safety and efficacy of AMX0035 in an international population of approximately 600 patients and build upon findings from the CENTAUR trial. Amylyx is currently exploring the possibility for an Expanded Access Program (EAP) in the United States. If implemented, the EAP would run in parallel with the ongoing Phase 3 PHOENIX trial and marketing application review. Further information about the EAP is expected in fourth quarter 2021.

About AMX0035
AMX0035 is an investigational product comprised of two complementary active agents, sodium phenylbutyrate (PB) and taurursodiol (TURSO; also known as ursodoxicoltaurine), which were combined in a co-formulation 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.

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