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AMYLYX PHARMACEUTICALS ANNOUNCES COMPLETION OF ENROLLMENT IN GLOBAL PHASE 3 PHOENIX TRIAL OF AMX0035 IN ALS

Topline results expected in 2024

Amylyx Pharmaceuticals announced the completion of enrollment in PHOENIX, a global, 48-week, randomized, placebo-controlled Phase 3 clinical trial of AMX0035 (sodium phenylbutyrate and taurursodiol [also known as ursodoxicoltaurine]) in people living with amyotrophic lateral sclerosis (ALS). Amylyx anticipates topline results in 2024. The study enrolled 664 participants living with ALS.

“The Phase 3 PHOENIX trial was an excellent collaboration between European and United States Centers for excellence in ALS research and care. We anticipate that PHOENIX will help us generate further data about the safety and efficacy of AMX0035,” said Leonard H. van den Berg, MD, PhD, Professor of Neurology at UMC Utrecht in the Netherlands and Chairman of the Treatment Research Initiative to Cure ALS (TRICALS).

“We are pleased to share this milestone from the PHOENIX trial, a collaborative effort to advance our scientific understanding of ALS and build upon the positive data from our Phase 2 CENTAUR trial,” added Sabrina Paganoni, MD, PhD, principal investigator of the CENTAUR study, investigator at the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital, and member of the Executive Committee of the Northeast Amyotrophic Lateral Sclerosis Consortium (NEALS).

The primary efficacy outcome of PHOENIX will be a joint assessment of Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) total score progression over 48 weeks, adjusted for mortality. Safety and tolerability will be assessed over 48 weeks.

Participants were randomized 3:2 to receive either AMX0035 or placebo for a 48-week period. PHOENIX spans more than 65 sites across Europe and the U.S., the majority of which are members of the NEALS or TRICALS consortia.

European participants completing the 48-week trial have the option to enroll in an open label extension (OLE) phase. During this phase, all participants receive AMX0035, and continued safety and efficacy measures will be assessed.

The design of PHOENIX was informed by the results of the Phase 2 CENTAUR clinical trial of AMX0035, which demonstrated a statistically significant benefit in function, as well as an observed benefit on survival in a longer-term post hoc analysis. Overall, reported rates of adverse events and discontinuations in CENTAUR 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.

“We are grateful for the people living with ALS and their families who are participating in PHOENIX and the dedication of the study investigators,” said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. “We remain committed to continuing research and exploring the full potential of AMX0035 as part of our mission to one day end the suffering caused by ALS and other neurodegenerative diseases.”

EMA Marketing Application Review

The European Medicines Agency (EMA) is reviewing the submission of the Company’s Marketing Authorisation Application for AMX0035 for the treatment of ALS in Europe. The Company anticipates a decision from the EMA in the first half of 2023. It is expected that the ongoing Phase 3 PHOENIX clinical trial will be part of the obligations of the conditional marketing authorization if granted.

About AMX0035

AMX0035 is an oral, fixed-dose medication approved to treat amyotrophic lateral sclerosis (ALS) in adults in the U.S. as RELYVRIO™ (sodium phenylbutyrate and taurursodiol) and approved with conditions in Canada as ALBRIOZA™ (sodium phenylbutyrate and ursodoxicoltaurine). Additionally, the European Medicines Agency (EMA) is reviewing the Company’s Marketing Authorisation Application for AMX0035 for the treatment of ALS in Europe.

Source: **website Amylyx**