



Amylyx Pharmaceuticals to Present Updates on its AMX0114 and RELYVRIO® Programs in ALS at the 2024 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference

February 15, 2024 at 9:00 AM EST

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 15, 2024-- [Amylyx Pharmaceuticals, Inc.](#) (NASDAQ: AMLX) ("Amylyx" or the "Company") today announced that two abstracts detailing next steps in the development of the Company's investigational antisense oligonucleotide (ASO), AMX0114, for the potential treatment of amyotrophic lateral sclerosis (ALS) and findings from a pharmacokinetic and pharmacodynamic study of AMX0035 (sodium phenylbutyrate [PB] and taurursodiol [TURSO]) in people with ALS will be presented at the Muscular Dystrophy Association (MDA) Clinical and Scientific Conference on March 3-6, 2024 in Orlando, Florida. AMX0035 is marketed by Amylyx as RELYVRIO® and is approved to treat ALS in adults in the U.S. and approved with conditions as ALBRIOZA™ for the treatment of ALS in Canada.

Details of the poster presentations are as follows:

Title: Next Steps in Development for AMX0114: An Antisense Oligonucleotide Targeting Calpain-2, a Critical Effector of Axonal Degeneration
The Company is advancing AMX0114, our internally developed ASO targeting calpain-2, a critical effector of axonal degeneration in ALS and other neurodegenerative diseases, through investigational new drug enabling studies, with the goal of entering the clinic in 2024. This poster shares details on pre-clinical efficacy studies as well as introduces the design approach for the first-in-human study, anticipated to begin later this year. The Company is studying multiple cellular pathways implicated in disease pathogenesis as we believe that it is going to take a combination approach to find a cure for ALS.

Date and Time: March 4, 2024, from 6:00PM – 8:00PM EST

Title: Findings From a Pharmacokinetic and Pharmacodynamic Study of Sodium Phenylbutyrate and Taurursodiol in Participants With Amyotrophic Lateral Sclerosis

This poster provides results from a Phase 2a, open-label, sequential period study of AMX0035 pharmacokinetics in participants with ALS and includes safety data in addition to pharmacokinetics of AMX0035 and its major metabolites. No new safety signals were identified during this study.

Date and Time: March 4, 2024, from 6:00PM – 8:00PM EST

For conference information, visit: <https://www.mdaconference.org/>

Additional information, including copies of the poster presentations, will be made available on the "[Publications](#)" tab of the Amylyx website, following the conclusion of the poster presentations.

About RELYVRIO®/ ALBRIOZA™ / ALBRIOZA® / AMX0035

RELYVRIO®, an oral, fixed-dose combination of sodium phenylbutyrate and taurursodiol (known as ursodoxicoltaurine outside of the U.S.), is approved to treat amyotrophic lateral sclerosis (ALS) in adults in the U.S. and approved with conditions as ALBRIOZA™ for the treatment of ALS in Canada. AMX0035 is being studied for the potential treatment of other neurodegenerative diseases, and Amylyx is exploring its treatment in other populations and regions. The formulation of RELYVRIO, ALBRIOZA, and AMX0035 are identical.

RELYVRIO® (sodium phenylbutyrate and taurursodiol) Safety Information for United States

WARNINGS AND PRECAUTIONS

Risk in Patients with Enterohepatic Circulation Disorders, Pancreatic Disorders, or Intestinal Disorders

RELYVRIO contains taurursodiol, which is a bile acid. In patients with disorders that interfere with bile acid circulation, there may be an increased risk for worsening diarrhea, and patients should be monitored appropriately for this adverse reaction. Pancreatic insufficiency, intestinal malabsorption, or intestinal diseases that may alter the concentration of bile acids may also lead to decreased absorption of either of the components of RELYVRIO. Because different enterohepatic circulation, pancreatic, and intestinal disorders have varying degrees of severity, consider consulting with a specialist. Patients with disorders of enterohepatic circulation (e.g., biliary infection, active cholecystitis), severe pancreatic disorders (e.g., pancreatitis), and intestinal disorders that may alter concentrations of bile acids (e.g., ileal resection, regional ileitis) were excluded from the study; therefore, there is no clinical experience in these conditions.

Use in Patients Sensitive to High Sodium Intake

RELYVRIO has a high salt content. Each initial daily dosage of 1 packet contains 464 mg of sodium; each maintenance dosage of 2 packets daily contains 928 mg of sodium. In patients sensitive to salt intake (e.g., those with heart failure, hypertension, or renal impairment), consider the amount of daily sodium intake in each dose of RELYVRIO and monitor appropriately.

ADVERSE REACTIONS

The most common adverse reactions (at least 15% and at least 5% greater than placebo) with RELYVRIO were diarrhea, abdominal pain, nausea, and upper respiratory tract infection. Gastrointestinal-related adverse reactions occurred throughout the study but were more frequent during the first 3 weeks of treatment.

Please click [here](#) for RELYVRIO Full U.S. Prescribing Information.

About Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is committed to supporting and creating more moments for the neurodegenerative community through the discovery and development of innovative new treatments. Amylyx is headquartered in Cambridge, Massachusetts and has operations in Canada, EMEA, and Japan. For more information, visit [amylyx.com](https://www.amylyx.com) and follow us on [LinkedIn](#) and [X](#), formerly known as Twitter. For investors, please visit 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, the potential of AMX0114 as a treatment for ALS and the Company’s plans to develop AMX0114, the potential of AMX0035 as a treatment for ALS and the Company’s plans to explore the use of AMX0035 for other neurodegenerative diseases including PSP, the timelines for the ORION study in PSP, 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: Amylyx’ ability to fund operations, 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 and outcome of EMA’s review of AMX0035 for the treatment of ALS, Amylyx’ reliance on third parties, including to conduct clinical trials and manufacture products, and the effect of global economic uncertainty and financial market volatility caused by economic effects of rising inflation and interest rates, the COVID-19 pandemic, geopolitical instability, changes in international trade relationships and military conflicts, as well as the risks and uncertainties set forth in Amylyx’ United States Securities and Exchange Commission (SEC) filings, including Amylyx’ Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, 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. Subject to any obligations under applicable law, 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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