RELYVRIO® and AMX0114 Data to be Presented at 34th International Symposium on ALS/MND

November 27, 2023 at 9:00 AM EST

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 27, 2023-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) (“Amylyx” or the “Company”) today announced that several abstracts detailing data on AMX0035 (sodium phenylbutyrate and taurursodiol [PB&TURSO]) and the Company’s investigational antisense oligonucleotide, AMX0114, for the potential treatment of amyotrophic lateral sclerosis (ALS) and other neurodegenerative diseases will be presented at the 34th International Symposium on ALS/MND. New data using a model for assessing mortality-adjusted progression (MAP) for clinical trials of ALS and encore posters will be presented.

Details of the poster presentations are as follows:

Wednesday, December 6, 2023, Session A, 5:45 – 7:30 p.m. CET

- **Title:** A Joint Model for Assessing Mortality-Adjusted Progression (MAP) in Amyotrophic Lateral Sclerosis: Application to Clinical Trials of Sodium Phenylbutyrate and Taurursodiol
  This poster features an overview of the MAP model, a method that has been developed by leading ALS clinicians and statisticians and detailed in previous publications as an approach to account for deaths, while providing an interpretable and clinically relevant ALS Functional Rating Scale–Revised (ALSFRS-R) result. Results from a post hoc analysis of the Phase 2 CENTAUR trial using this model are presented, which resulted in a nearly identical statistical outcome with increased precision as the primary analysis. The Company intends to use a similar model for the primary efficacy analysis in the ongoing Phase 3 PHOENIX trial, with results anticipated in the second quarter of 2024.
  Poster Number: CLT-30

- **Title:** Ongoing and Planned Studies to Further Elucidate the Efficacy, Safety, and Pharmacokinetics of Sodium Phenylbutyrate and Taurursodiol in Amyotrophic Lateral Sclerosis *(encore)*
  This poster outlines studies currently underway that further assess efficacy and safety of AMX0035 in people living with ALS, including in real-world settings.
  Poster Number: CLT-10

- **Title:** Development of a Composite Diagnostic Biomarker for Amyotrophic Lateral Sclerosis: Experimental Approach and Progress to Date *(encore)*
  One of the key drivers of diagnostic delay in ALS is the lack of reliable, validated biomarkers to aid in diagnosis. This poster provides an update on the progress made in developing a biomarker test that could make the diagnosis of ALS easier.
  Poster Number: BIO-14

Thursday, December 7, 2023, Session B, 5:45 – 7:45 p.m. CET

- **Title:** Real-World Experience and Strategies to Enhance the Palatability of the Combination Sodium Phenylbutyrate and Taurursodiol for the Treatment of Amyotrophic Lateral Sclerosis *(encore)*
  Sodium phenylbutyrate and taurursodiol can have a bitter taste to some people living with ALS, but the combination is generally well-tolerated with an acceptable safety profile. Surveying people living with ALS in the U.S. prescribed sodium phenylbutyrate and taurursodiol, this poster provides information on what we learned about their real-world experiences related to product taste.
  Poster Number: CW-06

Thursday, December 7, 2023, Session B, Part 1, 5:45 – 6:45 p.m. CET

- **Title:** Preliminary Experience With Sodium Phenylbutyrate and Taurursodiol in a United States Expanded Access Program *(encore)*
  In 2022, we completed the largest single-product ALS Expanded Access Program (EAP) in the U.S. to date to provide pre-approval access to sodium phenylbutyrate and taurursodiol to people living with ALS alongside the ongoing Phase 3 PHOENIX trial. This poster provides initial safety data and learnings gathered through this program.
  Poster Number: CLT-11

Thursday, December 7, 2023, Session B, Part 2, 6:45 – 7:45 p.m. CET

- **Title:** Update on AMX0114: An Antisense Oligonucleotide Targeting Calpain-2, a Critical Effector of Axonal Degeneration

(encore)
We believe that it is going to take a combination approach, targeting multiple cellular pathways implicated in disease pathogenesis, to find a cure for ALS. This poster provides an update on AMX0114, our internally developed antisense oligonucleotide (ASO) targeting calpain-2, a critical effector of axonal degeneration in ALS and other neurodegenerative diseases.
Poster Number: TST-12

For conference information, visit: https://symposium.mndassociation.org/

A copy of the abstract discussing the MAP model is published online in *Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration*. 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™ /AMX0035

RELYVRIO® (also known as AMX0035), 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 as an investigational drug in several other regions for the potential treatment of ALS and other neurodegenerative diseases. 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 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 functional 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, 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 & Nerve*.

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 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 and EMEA. For more information, visit 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 and related comments in our earnings conference call 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 AMX0035 (sodium phenylbutyrate and taurursodiol) as a treatment for ALS and other neurodegenerative diseases including Wolfram syndrome and PSP; the ongoing commercialization of RELYVRIO and ALBRIOZA; expectations

regarding the timing of initiation of the Company's Phase 3 ORION trial of AMX0035 for the treatment of PSP and of the results of the Company's Phase 2 HELIOS trial of AMX0035 for the treatment of Wolfram syndrome; the timing of the results of the Company's Phase 3 PHOENIX trial of AMX0035 for the treatment of ALS; expectations regarding the results of the Phase 3 PHOENIX trial and of the potential for future approval of AMX0035 in the EU; the timing of filing IND applications for AMX0114 and for a new formation of RELYVRIO; the potential for new pipeline programs and clinical indications for AMX0035; statements regarding regulatory developments; the Company's expectations with respect to its progress through IND enabling studies of AMX0114 and other advancements in its pipeline; the Company's expectations regarding its financial performance; and expectations regarding the Company's longer-term strategy. Any forward-looking statements in this press release and related comments in the Company's earnings conference call 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 successfully commercialize RELYVRIO in the United States and ALBRIOZA in Canada; Amylyx' ability to execute on its commercial and regulatory strategy; that data from later-stage trials may not reflect data from earlier-stage trials, including other indications; regulatory developments; Amylyx' ability to fund operations, and the impact that global macroeconomic uncertainty, geopolitical instability and public health events, such as COVID-19, will have on Amylyx' operations, 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 and related comments in our earnings conference call 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, except as required by law.

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