RELYVRIO® and AMX0114 Data to be Presented at 22nd Annual Northeast ALS Consortium (NEALS) Meeting

October 2, 2023 at 9:00 AM EDT

- Topics include safety data from the AMX0035 U.S. Expanded Access Program, progress on the development of an ALS diagnostic biomarker test, new *in vitro* data on AMX0114, and more

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 2, 2023-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) (“Amylyx” or the “Company”) today announced the acceptance of several abstracts for presentation at the hybrid 2023 Northeast ALS Consortium (NEALS) Annual Meeting. The data being shared are related to 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. 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. The in-person component of the NEALS Annual Meeting will take place in Clearwater, Florida October 4-6, 2023.

“We look forward to discussing updates from our research initiatives, in collaboration with the ALS community, as part of our collective efforts to contribute to a deeper understanding of ALS and the treatment and care paradigm,” said Machelle Manuel, PhD, Head, Global Medical Affairs at Amylyx. “There is still much to learn about ALS, and research is a critical pillar of how we’ll deliver on our mission to one day end the suffering caused by neurodegenerative diseases.”

Details of the poster presentations at the NEALS Annual Meeting are as follows:

Wednesday, October 4, 2023, 5:15 pm – 7:15 pm ET

- **Title:** Development of a Composite Diagnostic Biomarker for ALS: Experimental Approach and Progress to Date
  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: 38

- **Title:** Update on AMX0114: An Antisense Oligonucleotide Targeting Calpain-2, a Critical Effector of Axonal Degeneration
  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: 14

- **Title:** Novel Neuroprotective Strategies in Human Neuron Models of ALS/FTD: Evaluating Antisense Oligonucleotide Therapies, including AMX0114
  This poster details findings from a collaboration with Dr. Sami Barmada and his team at the University of Michigan School of Medicine in which the impact of ASOs targeting calpain-2, including AMX0114, on survival were evaluated in human iPSC-derived motor neurons harboring the ALS-linked TDP43^{M337V} mutation.
  Poster Number: 15

Thursday, October 5, 2023, 4:30 pm – 6:30 pm ET

- **Title:** Real-World Experience and Strategies to Enhance the Palatability of the Combination Sodium Phenylbutyrate and Taurursodiol for the Treatment of Amyotrophic Lateral Sclerosis
  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: 90

- **Title:** Preliminary Experience with Sodium Phenylbutyrate & Taurursodiol in a US Expanded Access Program
  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: 123

- **Title:** An *In Vitro* Recovery Study of Sodium Phenylbutyrate and Taurursodiol From 3 Types of Dosing Containers and Various Percutaneous Endoscopic Gastrostomy Feeding Tubes
  This poster details results of an in vitro study evaluating the use of sodium phenylbutyrate and taurursodiol with different types of feeding tubes and containers.
  Poster Number: 124

- **Title:** Ongoing and Planned Studies to Further Elucidate the Efficacy, Safety, and Pharmacokinetics of Sodium Phenylbutyrate and Taurursodiol in Amyotrophic Lateral Sclerosis
  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: 125

For conference information, visit: https://neals.org/als-researchers/annual-neals-meeting

Information about the 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®, 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 explored in other populations and regions, as well as for the potential treatment of 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 ALS

ALS is a relentlessly progressive and fatal neurodegenerative disorder caused by motor neuron death in the brain and spinal cord. Motor neuron loss in ALS leads to deteriorating muscle function, the inability to move and speak, respiratory paralysis and eventually, death. More than 90% of people with ALS have sporadic disease, showing no clear family history. ALS affects approximately 29,000 people in the U.S. and more than 30,000 people are estimated to be living with ALS in Europe (European Union and United Kingdom). People living with ALS have a median survival of approximately two years from diagnosis.

About Amylyx Pharmaceuticals

Amylyx Pharmaceuticals, Inc. is committed to supporting and creating more moments for the neurodegenerative disease 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 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 approvability of AMX0035 for conditional marketing authorisation in Europe; the ability to provide post-authorisation confirmatory data; our plans to make AMX0035 available in Europe; the ongoing commercialization of RELYVRIO and ALBRIOZA; the potential continued market acceptance and market opportunity for RELYVRIO and ALBRIOZA; the potential of AMX0035 as a treatment for ALS and the Company's plans to explore the use of AMX0035 for other

neurodegenerative diseases; the potential of other investigational treatments to be developed to treat ALS or other neurodegenerative diseases; 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 June 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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