Amylyx Pharmaceuticals Provides Update on Accès Compassionnel for AMX0035 in France

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 5, 2023-- L'Agence Nationale de Sécurité du Médicament et des produits de santé (ANSM), the national authority for authorizing medicinal products in France, has granted "accès compassionnel" (compassionate access) to AMX0035 (sodium phenylbutyrate and ursodoxicoltaurine [also known as taurursodiol]) for eligible people living with amyotrophic lateral sclerosis (ALS) following a collaborative process involving medical experts, ARSLA and other ALS advocates, ANSM, and Amylyx. AMX0035 is known as RELYVRIO® in the United States and ALBRIOZA™ in Canada. For more information, including eligibility criteria, please visit here.

Separately, the formal re-examination procedure of the Marketing Authorisation Application of AMX0035 for the treatment of adults with ALS in the European Union (EU) remains underway. Amylyx continues to engage with the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) to prioritize broad and sustainable access to AMX0035 through the central EU review and approval process.

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.

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. 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.eu. 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, statements regarding the timing and results of the global Phase 3 PHOENIX trial; the potential approval of AMX0035 for the treatment of ALS in countries other than the United States and Canada; the potential availability of AMX0035 in countries other than the United States and Canada through managed access programs; 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 process and timing of the EMA's formal re-examination procedure of the Company's Marketing Authorisation Application for AMX0035 for the treatment of ALS in Europe; 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 outcome of the re-examination of AMX0035 for the treatment of ALS, and 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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