Amylyx Pharmaceuticals Announces Health Canada Approval of ALBRIOZA™ for the Treatment of ALS

June 13, 2022

- Health Canada decision signifies the first global regulatory approval for Amylyx and the first new therapy for ALS approved in Canada since 2018
- ALBRIOZA (also known as AMX0035) is an oral fixed-dose combination therapy that may reduce neuronal cell death as a stand-alone therapy or when added to existing treatments
- In a clinical trial, ALBRIOZA significantly slowed disease progression and loss of functional decline in people living with ALS
- Detailed data from the CENTAUR clinical trial were published in the *New England Journal of Medicine, Muscle & Nerve,* and the *Journal of Neurology, Neurosurgery and Psychiatry*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jun. 13, 2022-- Amylyx Pharmaceuticals, Inc. (NASDAQ: AMLX) ("Amylyx" or the "Company") today announced that Health Canada has approved ALBRIOZA™ (sodium phenylbutyrate and ursodoxicoltaurine), with conditions, for the treatment of amyotrophic lateral sclerosis (ALS). Clinical data demonstrated a statistically significant and clinically meaningful benefit in functional outcomes for people with ALS taking ALBRIOZA (also known as AMX0035) compared to people taking placebo, either as a stand-alone therapy or when added to existing treatments for ALS. This decision marks the first marketing approval for ALBRIOZA issued to Amylyx worldwide.

"For nearly a decade, we have been committed to creating more meaningful moments for people living with ALS and their families. We are excited with Health Canada's decision to approve ALBRIOZA with conditions. ALBRIOZA is a therapy that demonstrated in our CENTAUR trial a statistically significant and clinically meaningful impact on function, alone or in addition to existing ALS therapies. We are grateful to the people who participated in our clinical trials, their families, the researchers, and the ALS community for helping to make this milestone happen," said Justin Klee and Joshua Cohen, Co-CEOs and Co-Founders of Amylyx.

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. The vast majority of people with ALS (>90%) have sporadic disease, showing no clear family history. Approximately 3,000 Canadians are currently living with ALS, and the average life expectancy from symptom onset is two to five years. Approximately 1,000 people die from ALS in Canada every year, with a similar number of diagnoses annually.

"I have always had a love for life and a passion for global advocacy. Thanks to new innovations in ALS treatment and care, I've been able to continue my advocacy work in new ways as part of the fight for new treatment options for the ALS community," said Norman MacIsaac, who has been living with ALS since 2014. "New therapies like ALBRIOZA are critical to help me and others have a fuller life. The ability to live more independently for longer is incredibly meaningful."

"For more than 30 years, I have been involved with caring for people living with ALS, and we have had major breakthroughs in research and made strides in drug development. The approval of ALBRIOZA is a testament to this progress," said Angela Genge, MD, FRCP(C), Director of the ALS Global Centre for Excellence at the Montreal Neurological Institute. "New treatment options that can slow disease progression are critical to give people living with ALS more time as we work toward a cure."

The approval of ALBRIOZA was authorized under Health Canada's Notice of Compliance with Conditions (NOC/c) policy. One of the conditions of the approval is the provision of data from the ongoing Phase 3 PHOENIX trial. Other conditions include additional planned or ongoing studies. Amylyx anticipates topline results from the PHOENIX trial in 2024.

The approval of ALBRIOZA is based on data from CENTAUR, 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, which demonstrated that participants treated with ALBRIOZA scored, on average, 2.32 points higher on the Amyotrophic Lateral Sclerosis Functional Rating Scale – Revised (ALSFRS-R) over a period of 24 weeks. Detailed data from CENTAUR were published in the *New England Journal of Medicine*, *Muscle & Nerve,* and the *Journal of Neurology, Neurosurgery, and Psychiatry*.

"We are delighted that this Health Canada approval represents the advancement in available treatment options for people living with ALS, but there is still more work to be done," said Tammy Moore, CEO of the ALS Society of Canada. "ALS is a devastating disease and can move with startling swiftness. It is incredibly important that all Canadians across the country are able to benefit from these and other innovations to come, as quickly as possible following regulatory approval. We are hopeful that decision-

makers throughout the drug access and reimbursement process will work expeditiously to provide timely and equitable access for all Canadians who may benefit. There is simply no time to wait with this disease."

"The approval by Health Canada, the first for Amylyx globally, is an important milestone and first step," said Chris Aiello, General Manager and Head of Canada at Amylyx. "We anticipate that ALBRIOZA will be available commercially in Canada within the next six weeks. We are prepared to work with the pan-Canadian Pharmaceutical Alliance (pCPA) and federal, provincial and territorial governments to negotiate the listing of ALBRIOZA expeditiously, so that eligible Canadians living with ALS can obtain access as quickly and efficiently as possible."

As part of Amylyx' continued commitment to the ALS community, the Amylyx Care Team (ACT) Support Program provides insurance navigation, treatment coordination and educational support for people living with ALS who have been prescribed ALBRIOZA and their caregivers. Eligibility and enrolment into the ACT Support Program can be discussed with prescribing health care professionals.

Marketing Application Reviews & Clinical Update

AMX0035 has been granted Priority Review by the U.S. Food and Drug Administration (FDA) and assigned a Prescription Drug User Fee Act date of September 29, 2022. Additionally, The European Medicines Agency (EMA) has validated the submission of the Company's Marketing Authorisation Application for AMX0035 for the treatment of ALS in Europe and it is under review. A 48-week, randomized placebo-controlled Phase 3 PHOENIX (NCT05021536) trial of AMX0035 in more than 65 sites across the U.S. and Europe is underway.

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 Amyotrophic Lateral Sclerosis 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, GI events occurred with greater frequency (≥2%) in the AMX0035 group. Detailed safety and functional efficacy data from CENTAUR were published in the *New England Journal of Medicine*. Data from additional analyses from the CENTAUR trial were published in *Muscle & Nerve* in 2020 and 2022, and the *Journal of Neurology, Neurosurgery and Psychiatry* in 2022.

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 ALBRIOZA™

ALBRIOZA™ (sodium phenylbutyrate and taurursodiol) is an oral fixed-dose medication approved with conditions to treat amyotrophic lateral sclerosis (ALS) in Canada. The combination of sodium phenylbutyrate and taurursodiol may reduce neuronal cell death, hypothesized to occur by simultaneously mitigating endoplasmic reticulum (ER) stress and mitochondrial dysfunction. ALBRIOZA is being explored for the potential treatment of other neurodegenerative diseases.

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 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, statements regarding the potential approval of AMX0035 for the treatment of ALS in countries other than Canada; the potential of AMX0035 as a treatment for ALS; the potential market acceptance and market opportunity for ALBRIOZA™; the Company's ability to conduct a confirmatory trial of AMX0035 as a treatment for ALS; the Company's ability to make ALBRIOZA available commercially in Canada, as well as access to and coverage for ALBRIOZA; and expectations regarding our longer-term strategy. Any forward-looking statements in this statement 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 launch ALBRIOZA in Canada, Amylyx' ability to execute on its commercial and regulatory strategy, regulatory developments, expectations regarding the timing of FDA and EMA review of AMX0035 for the treatment of ALS, Amylyx' ability to fund operations, and the impact that the ongoing COVID-19 pandemic 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' Annual Report on Form 10-K for the year ended December 31, 2021, 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. 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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