Amylyx Pharmaceuticals Reports Second Quarter 2022 Financial Results

August 11, 2022

- *ALBRIOZA™ (also known as AMX0035) now commercially available in Canada, representing first product launch for Amylyx*

- *FDA Advisory Committee meeting to review new drug application for AMX0035 for the treatment of ALS scheduled for September 7, 2022; assigned PDUFA action date of September 29, 2022; U.S. commercial team now launch ready*

- *Marketing Authorisation Application under review and preparations underway for commercialization of AMX0035 in the European Union*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 11, 2022-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial results for the quarter ended June 30, 2022.

"The second quarter of 2022 has been another exciting time for Amylyx, marked by our first regulatory approval. ALBRIOZA™ is now commercially available in Canada, signifying an important milestone on our path to helping people living with ALS," said Justin Klee, Co-CEO of Amylyx.

Josh Cohen, Co-CEO of Amylyx, added, "Looking ahead, we remain committed to advancing our ongoing regulatory interactions and preparing for launch in the U.S. and the European Union, if approved."

Second Quarter 2022 and Recent Business Highlights:

- **Announced Health Canada approval of ALBRIOZA (sodium phenylbutyrate and ursodoxicoltaurine), with conditions, for the treatment of amyotrophic lateral sclerosis (ALS).** This decision was the first marketing approval issued to Amylyx worldwide. Commercial launch of ALBRIOZA (also known as AMX0035) commenced on July 29, 2022, in Canada.

- **Regulatory applications under review for AMX0035 for the treatment of ALS in the U.S. and the European Union.**

    - *New Drug Application (NDA) for AMX0035 for the treatment of ALS under Priority Review by the U.S. Food and Drug Administration (FDA).* The FDA will reconvene the Peripheral and Central Nervous System Drugs Advisory Committee (PCNSDAC) on September 7, 2022, with discussions focused on the additional analyses of data from the Company's clinical studies that were determined by the FDA to constitute a major amendment to the NDA. The Prescription Drug User Fee Act (PDUFA) action date is September 29, 2022.

    - *Marketing Authorisation Application for AMX0035 for the treatment of ALS under review with European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP).* The review process is proceeding as expected, with receipt of the Day 120 List of Questions following the June CHMP meeting. Amylyx will continue to work with EMA through its review process, and the Company expects a decision in the first half of 2023. Preparations are underway for commercialization of AMX0035 in the European Union, if approval is received.

- **Announced planned open-label extension (OLE) phase for Phase 3 PHOENIX trial in ALS at the 17th International Congress on Neuromuscular Diseases (ICNMD 2022).** The poster included an overview of the global Phase 3 PHOENIX trial design and methodology, as well as an update on a planned OLE phase for PHOENIX, and was presented on July 9, 2022, in Brussels, Belgium.

- **Announced presentation of new analysis of CENTAUR trial results at the European Network to Cure ALS (ENCALS) 2022.** A new post hoc analysis measuring substantial individual response to AMX0035 compared to placebo continues to demonstrate the efficacy of the combination and was presented in Edinburgh, Scotland on June 2, 2022.

- **Announced publication of data showing lower occurrence of tracheostomy/permanent assisted ventilation (PAV) and delayed first hospitalization in peer-reviewed medical journal, *Journal of Neurology, Neurosurgery, and Psychiatry*.** The analyses of data from the Phase 2 CENTAUR trial showed that randomization to AMX0035 resulted in a lower risk of occurrence of death or tracheostomy/PAV by 49% and first hospitalization by 44% over the course of the Phase 2 trial and duration of follow-up. As of the analysis cutoff with the longest follow up of 35 months, median time to first hospitalization was not yet reached in the group originally randomized to AMX0035, compared with 14.1 months in the group originally randomized to placebo.

- **Announced publication of new analyses from the CENTAUR trial, further demonstrating significant survival benefit**

of AMX0035 in peer-reviewed medical journal, *Muscle & Nerve*. A post hoc analysis using the rank-preserving structural failure time model (RPSFTM), a method frequently employed in oncology to account for placebo crossover, estimated a 10.6-month longer median survival duration for participants originally randomized to AMX0035 than participants originally randomized to placebo. In addition, participants randomized to receive AMX0035 and who continued into the OLE phase showed an 18.8-month longer median survival duration than participants who never received AMX0035 in a subgroup analysis.

Financial Results for the Second Quarter Ended June 30, 2022

For the quarter ended June 30, 2022, research and development expenses were $24.3 million, compared to $10.9 million for the quarter ended June 30, 2021. The increase was mainly driven by higher product manufacturing and development expenses in support of clinical trial and launch preparation activities for commercialization, and increased personnel-related expenses due to increased headcount to support research and development efforts.

General and administrative expenses were $30.0 million for the quarter ended June 30, 2022, compared to $7.7 million for the quarter ended June 30, 2021. The increase was primarily due to higher personnel-related expenses due to increased headcount to support launch preparation initiatives and increased consulting and professional services fees for commercial readiness activities and operations as a public company.

Net loss for the quarter ended June 30, 2022, was $54.1 million, or $0.93 per share, compared to a net loss of $21.9 million, or $3.41 per share, for the same period in 2021.

Cash, cash equivalents and short-term investments were $206.7 million at June 30, 2022, compared to $255.2 million at March 31, 2022.

About AMX0035

AMX0035 (sodium phenylbutyrate and taurursodiol) is an oral fixed-dose medication approved with conditions as ALBRIOZA™ to treat amyotrophic lateral sclerosis (ALS) in Canada and with marketing applications pending in the United States and the European Union. 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. AMX0035 is also being explored for the potential treatment of other neurodegenerative diseases.

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, gastrointestinal 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 the PHOENIX Trial

The Phase 3 PHOENIX clinical trial (NCT05021536) is a 48-week, randomized placebo-controlled global clinical trial further evaluating the safety and efficacy of AMX0035 (sodium phenylbutyrate and taurursodiol) for the treatment of ALS. The primary efficacy outcome of the trial will be a composite measure of survival and Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) total score progression over 48 weeks and survival and tolerability over 48 weeks. Secondary endpoints include change in slow vital capacity (SVC), measured both at home using a self-administered spirometer to support virtual data collection and at clinic sites using standard spirometry, quality of life patient-reported outcome assessments, ventilation-free survival rates and other measures. More information on the PHOENIX trial can be found at www.clinicaltrials.gov and eudract.ema.europa.eu.

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: Amylyx' strategy, business plans and objectives for 2022 and beyond; the potential of AMX0035 as a treatment for ALS, the efficacy and safety profile of AMX0035 and the potential for regulatory approval of AMX0035 as a treatment for ALS in the U.S. and the European Union and to maintain approval in Canada; the commercialization of ALBRIOZA in Canada and potential commercial launch of AMX0035 as a treatment for ALS in other jurisdictions, if approved, and the ability to scale operations to prepare for commercial launch; the timing, progress and results of our global Phase 3 PHOENIX clinical trial of AMX0035 and potential OLE extension; the potential of AMX0035 or other future therapeutic candidates as a treatment for neurodegenerative diseases generally; 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 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, market acceptance of AMX0035, Amylyx' ability to fund operations, and the impact that the ongoing COVID-19 pandemic will have on Amylyx' operations, as well as those 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.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
UNAUDITED
(in thousands)

	June 30, 2022	December 31, 2021
Assets		
Cash, cash equivalents and short-term investments	$ 206,681	$ 96,118
Prepaid expenses and other current assets	9,458	5,392
Deferred offering costs	—	3,441
Other assets	9,101	663
Total assets	$ 225,240	$ 105,614
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Accounts payable and accrued expenses	$ 25,377	$ 17,396
Other liabilities	7,074	35
Total liabilities	32,451	17,431
Redeemable convertible preferred stock	—	239,351
Stockholders' equity (deficit)	192,789	(151,168)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 225,240	$ 105,614

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
UNAUDITED
(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 24,259	$ 10,929	$ 45,723	$ 17,793
General and administrative	29,994	7,658	56,344	13,662
Total operating expenses	54,253	18,587	102,067	31,455

Loss from operations	(54,253)	(18,587)	(102,067)	(31,455)
Other income (expense), net	360	(3,335)	472	(4,990)
Loss before income taxes	(53,893)	(21,922)	(101,595)	(36,445)
Provision for income taxes	174	—	320	—
Net loss	$ (54,067)	$ (21,922)	$ (101,915)	$ (36,445)
Net loss per share attributable to common stockholders —basic and diluted	$ (0.93)	$ (3.41)	$ (1.85)	$ (5.75)
Weighted-average shares used in computing net loss per share attributable to common stockholders—basic and diluted	58,275,903	6,433,889	54,958,537	6,334,813

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