Amylyx Pharmaceuticals Reports First Quarter 2023 Financial Results

May 11, 2023

- *First quarter 2023 product revenue of $71.4 million; commercial launches of RELYVRIO® in U.S. and ALBRIOZA™ inCanada for the treatment of ALS continue to progress*

- *Regulatory review ongoing in the EU*

- *Expansion of pipeline with first participant dosed in Phase 2 HELIOS study of AMX0035 in Wolfram syndrome and planned initiation of a pivotal Phase 3 study in progressive supranuclear palsy anticipated this year*

- *Management to host conference call and webcast today at 4:30 p.m. Eastern Time*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 11, 2023-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial results for the first quarter ended March 31, 2023.

"During the first quarter, we made significant progress on our commercial launches of RELYVRIO in the U.S. and ALBRIOZA in Canada as we advanced our goal of ensuring efficient access for every eligible person living with ALS. We continue to see strong engagement and interest from physicians and the ALS community and are encouraged that the vast majority of payors who have published formal policy decisions are providing broad access to RELYVRIO. We remain focused on our key commercial priorities to drive awareness, educate payors, and help people gain access to therapy as quickly as possible," said Joshua Cohen, co-CEO of Amylyx. "We also continue to engage with the European Medicines Agency as it reviews our Marketing Authorisation Application for AMX0035 and continue to expect a decision later this year."

Justin Klee, co-CEO of Amylyx, added, "We are pleased with our recent progress but understand there is much more work to be done to achieve our mission of one day ending the suffering caused by ALS and other neurodegenerative diseases. With this in mind, we are excited to announce our plans to initiate a new Phase 3 study of AMX0035 in progressive supranuclear palsy later this year, supported by findings from our PEGASUS Phase 2 study in Alzheimer's disease as well as a number of preclinical studies which indicate the potential for AMX0035 to lower levels of tau, a biomarker often seen in people with neurodegenerative diseases, including PSP."

First Quarter 2023 and Recent Business Highlights:

- **Commercial launches of RELYVRIO (sodium phenylbutyrate and taurursodiol) in the U.S. and ALBRIOZA (sodium phenylbutyrate and ursodoxicoltaurine) in Canada, previously known as AMX0035, continue to progress.** Net product revenue for the three months ended March 31, 2023 was $71.4 million, compared to net product revenue of $21.9 million for the three months ended December 31, 2022.

- **Marketing Authorisation Application (MAA) for AMX0035 for the treatment of ALS under review with the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP).** The Company continues to expect an opinion from the CHMP mid-year and a decision in the third quarter of 2023 at the earliest.

- **Announced completion of enrollment in global Phase 3 PHOENIX trial of AMX0035 in ALS.** The trial enrolled 664 adult participants living with ALS. Amylyx continues to expect topline results in mid-2024.

- **Announced the first participant has been dosed in the HELIOS study, a Phase 2 clinical trial of AMX0035 for the treatment of Wolfram syndrome.** HELIOS is an exploratory open-label proof of biology study assessing the effect of AMX0035, its safety and tolerability, and various measures of endocrinological, neurological, and ophthalmologic function in adults with Wolfram syndrome. Amylyx anticipates topline results from HELIOS in 2024.

- **Plans underway to initiate a global, pivotal Phase 3 trial of AMX0035 for the treatment of progressive supranuclear palsy (PSP).** PSP is a rare, progressive neurological disorder that affects body movements, walking and balance, and eye movement and is typically fatal within 5 to 8 years. There are currently no approved disease-modifying therapies for the treatment of PSP, and the disease affects between five and seven in 100,000 people worldwide. PSP is characterized by widespread neurodegeneration associated with tau protein deposition in subcortical regions of the brain. Based on preclinical data and biomarker analysis from the Phase 2 PEGASUS study of AMX0035 in Alzheimer's disease, AMX0035 was shown to lower levels of tau and other markers of neurodegeneration. The Company plans to initiate a global, pivotal Phase 3 trial this year and intends to enroll approximately 600 adult participants.

- **Entered into an exclusive license and distribution agreement with Israel-based Neopharm for AMX0035.** Neopharm will commercialize AMX0035 for the treatment of ALS, subject to regulatory review and approval, in Israel, Gaza, West

Bank, and the Palestinian Authority.

- **Expanded global commercial leadership team.** Amylyx appointed Masako Nakamura to General Manager and Head of International Markets - Asia Pacific and Latin America. Ms. Nakamura brings 30 years of commercial, general management, and operational leadership experience in the biopharmaceutical industry with a strong focus on introducing rare disease therapies worldwide across multiple therapeutic areas.

Financial Results for the First Quarter Ended March 31, 2023

For the three months ended March 31, 2023, net product revenue was $71.4 million and cost of sales were $5.3 million. Both net product revenue and cost of sales during the period were attributable to sales of RELYVRIO in the U.S. and ALBRIOZA in Canada. There were no product revenues or cost of sales for the comparable period in 2022.

Research and development expenses were $24.2 million for the three months ended March 31, 2023, compared to $21.5 million for the same period in 2022. The increase was mainly driven by an increase in personnel-related expenses due to added headcount to support research and development efforts.

Selling, general and administrative expenses were $44.0 million for the three months ended March 31, 2023, compared to $26.3 million for the same period in 2022. The increase was mainly driven by higher personnel-related expenses due to added headcount to support our growth, as well as commercialization initiatives.

Net income for the three months ended March 31, 2023, was $1.6 million, or $0.02 per share, compared to a net loss of $47.8 million, or $0.93 per share for the same period in 2022.

Cash, cash equivalents, and short-term investments were $345.7 million at March 31, 2023, compared to $346.9 million at December 31, 2022.

Investor Conference Call Information
Amylyx' management team will host a conference call and webcast today, May 11, 2023, at 4:30 p.m. ET to discuss financial results and provide an update on the business. To access the conference call, please dial (833) 816-1395 (U.S.) or +1 (412) 317-0488 (international) at least 10 minutes prior to the start time and ask to be joined into the Amylyx Pharmaceuticals call. A live audio webcast of the call will be available under "Events and Presentations" in the Investor section of the Company's website, https://investors.amylyx.com/news-events/events. The webcast will be archived and available for replay for 90 days following the event.

Available Information
We periodically provide other information for investors on our corporate website, https://www.amylyx.com, and our investor relations website, https://investors.amylyx.com. This includes press releases and other information about financial performance, information on corporate governance, and details related to our annual meeting of stockholders. We intend to use our website as a means of disclosing material non-public information and for complying with our disclosure obligations under Regulation FD. Accordingly, investors should monitor our website, in addition to following the Company's press releases, SEC filings, and public conference calls and webcasts.

About RELYVRIO®/ALBRIOZA™ /AMX0035

RELYVRIO®, an oral, fixed-dose medication 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. Additionally, the European Medicines Agency (EMA) is reviewing the Company's Marketing Authorisation Application for AMX0035 for the treatment of ALS in Europe. AMX0035 is being explored 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 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 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, statements regarding the potential approval of AMX0035 for the treatment of ALS in the European Union; the timing of an anticipated decision in Europe regarding whether to approve AMX0035 for the treatment of ALS; the potential of AMX0035 (sodium phenylbutyrate and taurursodiol) as a treatment for ALS and other neurodegenerative diseases including Wolfram syndrome and progressive supranuclear palsy; the ongoing commercialization of RELYVRIO and ALBRIOZA; the potential continued market acceptance and market opportunity for RELYVRIO and ALBRIOZA; statements regarding coverage and policy decisions by insurance plans related to RELYVRIO and ALBRIOZA and whether those decisions will be favorable; expectations regarding access to and coverage for RELYVRIO and ALBRIOZA; our ability to execute a successful launch in Europe, if approval is obtained; the Company’s expectations with respect to completion of and the timing of results from its ongoing PHOENIX trial; the potential for new pipeline programs and clinical indications for AMX0035; statements regarding regulatory developments, including those relating to the Company’s ongoing clinical trial of AMX0035 in Wolfram syndrome; the Company’s expectations with respect to its planned clinical trial of AMX0035 in patients with PSP; the anticipated commercialization of AMX0035 for the treatment of ALS, subject to regulatory review and approval, in Israel, Gaza, West Bank, and the Palestinian Authority; the anticipated benefits of strategic new hires; the Company’s expectations regarding its financial performance, the anticipated ramp in its R&D and SG&A expenses, gross-to-net revenue and its ability to achieve break even cash flow; 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, regulatory developments, expectations regarding the timing of EMA review of AMX0035 for the treatment of ALS, Amylyx’ ability to fund operations, and the impact that the ongoing COVID-19 pandemic, global macroeconomic uncertainty and geopolitical instability 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, 2022, 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.

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

	March 31, 2023	December 31, 2022
Assets		
Cash, cash equivalents and short-term investments	$ 345,674	$ 346,945
Prepaid expenses and other current assets	12,749	10,113
Accounts receivable, net	17,555	15,306
Inventories	23,148	9,769
Other assets	8,832	9,320

Total assets	$ 407,958	$ 391,453
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Accounts payable and accrued expenses	$ 49,523	$ 44,569
Other liabilities	5,787	6,277
Total liabilities	55,310	50,846
Stockholders' equity (deficit)	352,648	340,607
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 407,958	$ 391,453

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

	Three Months Ended March 31,	
	2023	**2022**
Product revenue, net	$ 71,428	$ —
Operating expenses:		
Cost of sales	5,283	—
Research and development	24,192	21,464
Selling, general and administrative	44,006	26,350
Total operating expenses	73,481	47,814
Loss from operations	(2,053)	(47,814)
Other income, net	3,456	112
Income (loss) before income taxes	1,403	(47,702)
(Benefit) provision for income taxes	(170)	146
Net income (loss)	$ 1,573	$ (47,848)
Net income (loss) per share attributable to common stockholders		
Basic	$ 0.02	$ (0.93)
Diluted	$ 0.02	$ (0.93)
Weighted-average shares used in computing net income (loss) per share attributable to common stockholders		
Basic	66,717,271	51,604,310
Diluted	70,863,665	51,604,310

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