Amylyx Pharmaceuticals Reports Fourth Quarter and Full Year 2022 Financial Results

March 13, 2023

- *Full year product revenue of $22.2 million; commercial launches of RELYVRIO® in U.S. and ALBRIOZA™ in Canada continue to progress*
- *Regulatory review ongoing in the EU*
- *Karen Firestone, CEO and co-founder of Aureus Asset Management and prior fund manager at Fidelity Investments, appointed to the Company's Board of Directors*
- *Management to host conference call and webcast today at 4:30 p.m. Eastern Time*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 13, 2023-- Amylyx Pharmaceuticals, Inc. (Nasdaq: AMLX) ("Amylyx" or the "Company") today reported financial results for the fourth quarter and full year ended December 31, 2022. The Company also announced the appointment of Karen Firestone, Chairman, CEO, and co-founder of Aureus Asset Management and prior fund manager at Fidelity Investments, to the Company's Board of Directors, effective March 16.

"2022 was an exceptionally exciting year for Amylyx, culminating with the approval of RELYVRIO in the U.S. following the approval with conditions for ALBRIOZA in Canada, marking an important step toward delivering much-needed advancements in treatment options to the ALS community," said Joshua Cohen and Justin Klee, Co-CEOs of Amylyx. "Our commercial launch is off to a strong start, and we are encouraged by the engagement we have seen from physicians, people living with ALS, and payors. While we are thrilled with this progress, we are also cognizant that much work remains to meet our goal of quick and efficient access for every eligible person living with ALS. With that in mind, we continue to engage with the European Medicines Agency as it reviews our Marketing Authorisation Application for AMX0035, and we remain focused on our efforts to engage stakeholders throughout the ALS community as we work to drive the broadest coverage possible for this important new therapeutic option."

Cohen and Klee continued, "We are also delighted to welcome Karen Firestone from Aureus Asset Management to our Board, and we look forward to her strategic guidance and perspectives as we continue to launch RELYVRIO and ALBRIOZA and focus on bringing innovative treatments to the neurodegenerative disease community."

"Amylyx has the potential to change the treatment landscape for people living with ALS, and I am delighted to join the Board at this pivotal time," said Ms. Firestone. "I look forward to collaborating with the talented team and leveraging my insights across risk management, corporate strategy, and financial performance in support of our mission to one day end the suffering caused by neurodegenerative diseases."

Full Year 2022 and Year-to-Date 2023 Business Highlights:

- **Announced FDA approval of RELYVRIO (sodium phenylbutyrate and taurursodiol), previously known as AMX0035 in the U.S., for the treatment of adults with amyotrophic lateral sclerosis (ALS).** Amylyx received U.S. FDA approval for RELYVRIO in September 2022 with the first commercial product being available in October 2022. This decision represented Amylyx' first regulatory approval of AMX0035 in the U.S. and its second worldwide.
- **Announced Health Canada approval of ALBRIOZA (sodium phenylbutyrate and ursodoxicoltaurine), previously known as AMX0035 in Canada, for the treatment of ALS.** Health Canada approved ALBRIOZA with conditions in June 2022, and it became available in Canada in July 2022, marking the first commercial availability of AMX0035 worldwide.
- **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).** Amylyx completed the Scientific Advisory Group meeting. Certain major objections remain, and the CHMP has adopted another round of questions as part of the regulatory process. Amylyx is now in possession of those questions. In order to respond in accordance with the updated timelines, the Company now expects an opinion from 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 in February 2023.** The trial has enrolled 664 adult participants living with ALS. Amylyx continues to expect topline data in mid-2024.
- **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.
- **Completed an upsized public offering.** In October 2022, the Company raised $230.6 million in net proceeds in a public offering of its common stock.
- **Strengthened Board of Directors.** Amylyx appointed Karen Firestone to the Board. Ms. Firestone brings more than three decades of biotechnology investment experience across Fidelity and Aureus Asset Management.

Financial Results for the Fourth Quarter and Year Ended December 31, 2022

For the three months ended December 31, 2022, net product revenue was $21.9 million and cost of sales were $2.8 million. For the year ended

December 31, 2022, net product revenue was $22.2 million and cost of sales were $3.0 million. Both net product revenue and cost of sales during these periods 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 periods in 2021.

Research and development expenses were $22.8 million for the three months ended December 31, 2022, compared to $13.4 million for the same period in 2021. Research and development expenses were $93.5 million for the year ended December 31, 2022, compared to $44.0 million for the same period in 2021. The increase was mainly driven by costs associated with the global Phase 3 PHOENIX clinical trial and its open label extension phase, commercial launch preparation activities, and additional personnel-related expenses due to new headcount to support research and development efforts.

Selling, general and administrative expenses were $40.8 million for the three months ended December 31, 2022, compared to $14.9 million for the same period in 2021. Selling, general and administrative expenses were $127.1 million for the year ended December 31, 2022, compared to $38.9 million for the same period in 2021. The increase was mainly driven by higher personnel-related expenses to support launch preparation initiatives as well as increased expenses for commercial launch activities and operations as a public company.

Net loss for the three months ended December 31, 2022 was $42.7 million, or $0.65 per share, compared to a net loss of $28.3 million, or $4.10 per share for the same period in 2021. Net loss for the year ended December 31, 2022, was $198.4 million, or $3.39 per share, compared to a net loss of $87.9 million, or $13.35 per share for the same period in 2021.

Cash, cash equivalents, and short-term investments were $346.9 million at December 31, 2022, compared to $162.6 million at September 30, 2022.

Investor Conference Call Information

Amylyx' management team will host a conference call and webcast today, March 13, 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://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™

RELYVRIO® (sodium phenylbutyrate and taurursodiol) is an oral, fixed-dose medication approved to treat amyotrophic lateral sclerosis (ALS) in adults in the U.S. and approved with conditions as ALBRIOZA™ (sodium phenylbutyrate and ursodoxicoltaurine) 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.

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 sodium phenylbutyrate and taurursodiol 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 and our ability to resolve remaining major objections as part of the MAA review process; the potential of sodium phenylbutyrate and taurursodiol as a treatment for ALS and other neurodegenerative diseases; the commercialization of RELYVRIO and ALBRIOZA, including our progress with uptake, enrollment and outreach and our expectations with respect to the number of patients on RELYVRIO and ALBRIOZA; the potential 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 regarding its financial performance, the anticipated ramp in our 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 our 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 successfully resolve outstanding issues with the review of its MAA, 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' Quarterly Report on Form 10-Q for the quarter ended September 30, 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)

	December 31,	
	2022	**2021**
Assets		
Cash, cash equivalents and short-term investments	$ 346,945	$ 96,118
Prepaid expenses and other current assets	10,113	5,392
Accounts receivable, net	15,306	—
Inventories	9,769	—
Deferred offering costs	—	3,441
Other assets	9,320	663
Total assets	$ 391,453	$ 105,614

Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Accounts payable, accrued expenses and other current liabilities	$ 46,609	$ 17,396
Other liabilities	4,237	35
Total liabilities	50,846	17,431
Redeemable convertible preferred stock	—	239,351
Stockholders' equity (deficit)	340,607	(151,168)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 391,453	$ 105,614

AMYLYX PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

UNAUDITED

(in thousands, except share and per share data)

	Three Months Ended December 31,		Year Ended December 31,	
	2022	**2021**	**2022**	**2021**
Revenues:				
Product revenue, net	$ 21,885	$ —	$ 22,230	$ —
Grant revenue	—	—	—	285
Total revenues	21,885	—	22,230	285
Operating expenses:				
Cost of sales	2,821	—	2,993	—
Research and development	22,813	13,394	93,450	44,040
Selling, general and administrative	40,844	14,921	127,128	38,933
Total operating expenses	66,478	28,315	223,571	82,973
Loss from operations	(44,593)	(28,315)	(201,341)	(82,688)
Other income (expense), net	2,468	(29)	3,740	(5,243)

Loss before income taxes	(42,125)	(28,344)	(197,601)	(87,931)
Provision for income taxes	579	—	774	—
Net loss	$ (42,704)	$ (28,344)	$ (198,375)	$ (87,931)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.65)	$ (4.10)	$ (3.39)	$ (13.35)
Weighted-average shares used in computing net loss per share attributable to common stockholders—basic and diluted	65,416,712	6,910,413	58,495,587	6,586,349

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