UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2024

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-41199

Amylyx Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**46-4600503**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
43 Thorndike St. Cambridge, Massachusetts	**02141**
(Address of principal executive offices)	**(Zip Code)**

(617) 682-0917

(Registrant's telephone number, including area code)

Not Applicable

(Former name, former address and former fiscal year, if changed since last report)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	AMLX	Nasdaq Global Select Stock Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☒
Non-accelerated filer	☐	Smaller reporting company	☒
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of October 31, 2024, the registrant had 68,547,860 shares of common stock, $0.0001 par value per share, outstanding.

AMYLYX PHARMACEUTICALS, INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2024

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From time to time, we may use our website or our LinkedIn profile at www.linkedin.com/company/amylyx to distribute material information. Our financial and other material information is routinely posted to and accessible on the Investors section of our website, available at www.amylyx.com. Investors are encouraged to review the Investors section of our website because we may post material information on that site that is not otherwise disseminated by us. Information that is contained in and can be accessed through our website or our LinkedIn page is not incorporated into, and does not form a part of, this Quarterly Report on Form 10-Q.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or Quarterly Report, contains forward-looking statements which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would” or the negative of these terms or other comparable terminology. These statements are not guarantees of future results or performance and involve substantial risks and uncertainties. Forward-looking statements in this Quarterly Report include, but are not limited to, express or implied statements about:

- our ability to obtain regulatory approvals of avexitide, AMX0035 in Wolfram syndrome, progressive supranuclear palsy, or PSP, or any other indications, or any other current or future product candidates;
- the timing, progress and results of our research and development activities, preclinical studies and clinical trials, including the anticipated Phase 3 clinical program for avexitide in post-bariatric hypoglycemia, or PBH, our Phase 2 clinical trial of AMX0035 for the treatment of Wolfram syndrome known as the HELIOS trial, and our Phase 2b/3 global clinical trial of AMX0035 for the treatment of PSP known as the ORION trial, as well as any other development efforts, preclinical studies and clinical trials for our current and any future product candidates;
- our ability to successfully commercialize and market our product candidates, if approved, and the timing of any commercialization and marketing efforts;
- our ability to contract with third-party suppliers, manufacturers and other service providers and their ability to perform adequately and to produce sufficient quantities of clinical and, if needed, commercial supplies;
- the market size, opportunity, demand and growth potential for our current and any future product candidates, if approved;
- our ability to build and maintain our own sales and marketing capabilities, or seek collaborative partners, to commercialize our current and any future product candidates, if approved;
- our ability to obtain funding for our operations;
- our ability to retain the continued service of our key executives and to identify, hire and retain additional qualified professionals;
- our ability to successfully complete our ongoing or planned clinical trials of AMX0035 and avexitide and to advance any other current or future product candidates into, and successfully complete, preclinical studies and clinical trials;
- our ability to successfully recruit and enroll suitable patients in our clinical trials;
- the timing or likelihood of the accomplishment of various scientific, clinical, regulatory filings and approvals and other product development objectives;
- the rate and degree of market acceptance of AMX0035, avexitide and any other current or future product candidates, if approved, by physicians, patients, third-party payors and others in the medical community;
- the implementation of our business model and strategic plans for our business, products, product candidates and technology;
- our ability to identify, evaluate, in-license and develop additional products or product candidates to complement our existing pipeline and ability to successfully incorporate acquired assets into our existing pipeline;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our products, product candidates and technology;
- developments relating to our competitors and our industry, including any regulatory developments;
- our estimates regarding expenses, revenue, capital requirements, cash runway and future needs for additional financing;
- fluctuations of our quarterly and annual operating results and the related effects on our stock price;
- the effect of global financial and economic conditions and geopolitical events, including heightened and fluctuating interest rates and inflation, foreign exchange fluctuations, the risk of economic slowdown or recession in the United States, instability in the banking system, overall market volatility in the United States, including as a result of, among

other factors, the ongoing war between Russia and Ukraine, the Israel-Hamas war and escalating conflict in the Middle East, including political unrest, or similar events, on our business; and

•other statements about future events, including those listed under the section titled “Risk Factors”.

Any forward-looking statements in this Quarterly Report reflect our current views with respect to future events and with respect to our future financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those described under the section titled “Risk Factors” and elsewhere in this Quarterly Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

All of our forward-looking statements are as of the date of this Quarterly Report only. In each case, actual results may differ materially from such forward-looking information. We can give no assurance that such expectations or forward-looking statements will prove to be correct. An occurrence of or any material adverse change in one or more of the risk factors or risks and uncertainties referred to in this Quarterly Report or included in our other public disclosures or our other periodic reports or other documents or filings filed with or furnished to the Securities and Exchange Commission, or the SEC, could materially and adversely affect our business, prospects, financial condition and results of operations. Except as required by law, we do not undertake or plan to update or revise any such forward-looking statements to reflect actual results, changes in plans, assumptions, estimates or projections or other circumstances affecting such forward-looking statements occurring after the date of this Quarterly Report, even if such results, changes or circumstances make it clear that any forward-looking information will not be realized. Any public statements or disclosures by us following this Quarterly Report that modify or impact any of the forward-looking statements contained in this Quarterly Report will be deemed to modify or supersede such statements in this Quarterly Report.

We may from time to time provide estimates, projections and other information concerning our industry, the general business environment, and the markets for certain diseases, including estimates regarding the potential size of those markets and the estimated incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events, circumstances or numbers, including actual disease prevalence rates and market size, may differ materially from the information reflected in this Quarterly Report. Unless otherwise expressly stated, we obtained this industry, business information, market data, prevalence information and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources, in some cases applying our own assumptions and analysis that may, in the future, prove not to have been accurate.

TRADEMARKS

Solely for convenience, our trademarks and trade names in this report are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that we will not assert, to the fullest extent under applicable law, our rights thereto.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)
(Unaudited)

	September 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 71,839	$ 170,201
Marketable securities	162,556	201,161
Accounts receivable, net	1,731	40,050
Inventories	—	38,323
Prepaid expenses and other current assets	9,137	14,931
Total current assets	245,263	464,666
Property and equipment, net	1,252	2,686
Restricted cash equivalents	1,441	719
Operating lease right-of-use assets	2,275	3,725
Long-term inventories	—	44,957
Other assets	482	701
Total assets	$ 250,713	$ 517,454
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,487	$ 22,061
Accrued expenses	50,456	57,724
Operating lease liabilities, current portion	1,972	2,257
Total current liabilities	53,915	82,042
Operating lease liabilities, net of current portion	595	1,980
Total liabilities	54,510	84,022
Commitments and contingencies (Note 11)		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized	—	—
Common stock, $0.0001 par value; 300,000,000 shares authorized; 68,545,508 and 67,707,432 shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively	7	7
Additional paid-in capital	764,701	738,177
Accumulated deficit	(569,146)	(304,949)
Accumulated other comprehensive income	641	197
Total stockholders' equity	196,203	433,432
Total liabilities and stockholders' equity	$ 250,713	$ 517,454

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Product revenue, net	$ 416	$ 102,693	$ 88,036	$ 272,337
Operating expenses:				
Cost of sales	—	5,218	5,953	16,081
Cost of sales - inventory impairment and loss on firm purchase commitments	809	—	118,680	—
Acquired in-process research and development	36,203	—	36,203	—
Research and development	21,237	30,037	81,192	83,273
Selling, general and administrative	17,828	48,718	97,234	136,115
Restructuring expenses	—	—	22,851	—
Total operating expenses	76,077	83,973	362,113	235,469
(Loss) income from operations	(75,661)	18,720	(274,077)	36,868
Other income, net:				
Interest income	3,098	4,179	11,493	11,784
Other expense, net	(141)	(488)	(1,371)	(831)
Total other income, net	2,957	3,691	10,122	10,953
(Loss) income before income taxes	(72,704)	22,411	(263,955)	47,821
Provision for income taxes	—	1,518	242	3,281
Net (loss) income	$ (72,704)	$ 20,893	$ (264,197)	$ 44,540
Net (loss) income per share				
Basic	$ (1.07)	$ 0.31	$ (3.89)	$ 0.66
Diluted	$ (1.07)	$ 0.30	$ (3.89)	$ 0.63
Weighted-average shares used in computing net (loss) income per share				
Basic	68,091,446	67,414,669	67,990,613	67,124,407
Diluted	68,091,446	69,748,547	67,990,613	70,143,659

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE (LOSS) INCOME
(in thousands)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Net (loss) income	$ (72,704)	$ 20,893	$ (264,197)	$ 44,540
Other comprehensive (loss) income				
Foreign currency translation gain (loss)	301	(138)	100	(78)
Net unrealized gain on available-for-sale securities	437	59	344	13
Other comprehensive (loss) income	738	(79)	444	(65)
Comprehensive (loss) income	$ (71,966)	$ 20,814	$ (263,753)	$ 44,475

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance as of December 31, 2023	67,707,432	$ 7	$ 738,177	$ 197	$ (304,949)	$ 433,432
Issuance of common stock upon exercise of stock options	49,618	—	147	—	—	147
Issuance of common stock upon vesting of RSUs	218,537	—	—	—	—	—
Stock-based compensation expense	—	—	9,924	—	—	9,924
Other comprehensive loss	—	—	—	(274)	—	(274)
Net loss	—	—	—	—	(118,793)	(118,793)
Balance as of March 31, 2024	67,975,587	$ 7	$ 748,248	$ (77)	$ (423,742)	$ 324,436
Issuance of common stock upon exercise of stock options	59,981	—	23	—	—	23
Issuance of common stock upon vesting of RSUs	43,489	—	—	—	—	—
Stock-based compensation expense	—	—	9,570	—	—	9,570
Other comprehensive loss	—	—	—	(20)	—	(20)
Net loss	—	—	—	—	(72,700)	(72,700)
Balance as of June 30, 2024	68,079,057	$ 7	$ 757,841	$ (97)	$ (496,442)	$ 261,309
Issuance of common stock upon exercise of stock options	36,799	—	57	—	—	57
Issuance of common stock upon vesting of RSUs	429,652	—	—	—	—	—
Stock-based compensation expense	—	—	6,803	—	—	6,803
Other comprehensive income	—	—	—	738	—	738
Net loss	—	—	—	—	(72,704)	(72,704)
Balance as of September 30, 2024	68,545,508	$ 7	$ 764,701	$ 641	$ (569,146)	$ 196,203

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance as of December 31, 2022	66,512,011	$ 7	$ 694,906	$ (86)	$ (354,220)	$ 340,607
Issuance of common stock upon exercise of stock options	451,298	—	2,777	—	—	2,777
Issuance of common stock upon vesting of RSUs	132,294	—	—	—	—	—
Stock-based compensation expense	—	—	7,580	—	—	7,580
Other comprehensive income	—	—	—	111	—	111
Net income	—	—	—	—	1,573	1,573
Balance as of March 31, 2023	67,095,603	$ 7	$ 705,263	$ 25	$ (352,647)	$ 352,648
Issuance of common stock upon exercise of stock options	226,138	—	1,687	—	—	1,687
Issuance of common stock upon vesting of RSUs	32,676	—	—	—	—	—
Stock-based compensation expense	—	—	10,053	—	—	10,053
Other comprehensive loss	—	—	—	(97)	—	(97)
Net income	—	—	—	—	22,074	22,074
Balance as of June 30, 2023	$ 67,354,417	$ 7	$ 717,003	$ (72)	$ (330,573)	$ 386,365
Issuance of common stock upon exercise of stock options	145,384	—	938	—	—	938
Issuance of common stock upon vesting of RSUs	5,325	—	—	—	—	—
Stock-based compensation expense	—	—	10,283	—	—	10,283
Other comprehensive loss	—	—	—	(79)	—	(79)
Net income	—	—	—	—	20,893	20,893
Balance as of September 30, 2023	67,505,126	$ 7	$ 728,224	$ (151)	$ (309,680)	$ 418,400

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2024	**2023**
Cash flows used in operating activities:		
Net (loss) income	$ (264,197)	$ 44,540
Adjustments to reconcile net (loss) income to net cash used in operating activities:		
Stock-based compensation expense	26,297	27,627
Depreciation expense	744	787
Accretion of investment discounts, net	(8,065)	(7,368)
Inventory impairment and loss on firm purchase commitments	118,680	—
Charge for purchase of IPR&D assets	36,203	—
Other non-cash items	827	—
Changes in operating assets and liabilities:		
Accounts receivable, net	38,319	(14,047)
Inventories	(9,253)	(46,648)
Interest receivable	303	370
Prepaid expenses and other current assets	5,496	(7,596)
Operating lease right-of-use assets	1,450	1,335
Other assets	216	(10)
Accounts payable	(20,574)	62
Accrued expenses	(33,415)	825
Operating lease liabilities	(1,670)	(1,509)
Net cash used in operating activities	(108,639)	(1,632)
Cash flows provided by investing activities:		
Purchases of property and equipment	(137)	(885)
Purchases of IPR&D assets, including transaction costs	(36,203)	—
Purchases of investments	(231,986)	(174,187)
Proceeds from maturities of marketable securities	279,000	246,200
Net cash provided by investing activities	10,674	71,128
Cash flows provided by financing activities:		
Follow-on offering costs paid	—	(136)
Proceeds from exercise of stock options and RSUs vesting	1,984	6,647
Withholding taxes paid on stock-based awards	(1,746)	(3,198)
Net cash provided by financing activities	238	3,313
Effect of exchange rate changes on cash, cash equivalents and restricted cash equivalents	87	(77)
Net (decrease) increase in cash, cash equivalents and restricted cash equivalents	(97,640)	72,732
Cash, cash equivalents and restricted cash equivalents, beginning of period	170,920	63,245
Cash, cash equivalents and restricted cash equivalents, end of period	$ 73,280	$ 135,977
Reconciliation of cash, cash equivalents and restricted cash equivalents:		
Cash and cash equivalents	$ 71,839	$ 135,258
Restricted cash equivalents	1,441	719
Total cash, cash equivalents and restricted cash equivalents:	$ 73,280	$ 135,977
Supplemental disclosure of cash flow information:		
Unrealized gain on marketable securities	$ 344	$ 13
Taxes withheld on stock-based awards included in accrued expenses	$ 54	$ 15
Purchases of property and equipment included in accounts payable	$ —	$ 150
Income taxes paid	$ 264	$ 4,978

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

AMYLYX PHARMACEUTICALS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(UNAUDITED)

1. NATURE OF THE BUSINESS

Amylyx Pharmaceuticals, Inc., together with its wholly owned subsidiaries, known as Amylyx or the Company, is a biotechnology company that is committed to the discovery and development of new treatment options for communities with high unmet needs, including people living with serious and fatal neurodegenerative diseases and endocrine conditions.

On July 9, 2024, Amylyx completed the acquisition of avexitide for the potential treatment of hyperinsulinemic hypoglycemia, a disease of the endocrine system. Avexitide is an investigational, first-in-class glucagon-like peptide-1, or GLP-1, receptor antagonist that has been evaluated in five clinical trials for post-bariatric hypoglycemia, or PBH, and has also been studied in congenital hyperinsulinism, or HI, two indications characterized by hyperinsulinemic hypoglycemia. The U.S. Food and Drug Administration, or FDA, has granted avexitide breakthrough therapy designation for both indications, rare pediatric disease designation in congenital HI, and orphan drug designation for the treatment of hyperinsulinemic hypoglycemia (which includes PBH and congenital HI). Amylyx expects to begin the Phase 3 program for avexitide in PBH in the first quarter of 2025.

The Company is also investigating AMX0035, developed by Amylyx, in both neurodegenerative and endocrine diseases where endoplasmic reticulum, or ER, stress and mitochondrial dysfunction are implicated, including progressive supranuclear palsy, or PSP, and Wolfram syndrome. The Company reported positive topline results from the Phase 2 open-label HELIOS clinical trial in 12 adults living with Wolfram syndrome in October 2024. The Company is engaging with stakeholders, including the FDA, to inform a Phase 3 program and expects to provide an update in 2025. The Company dosed the first participant in the ORION trial, a Phase 2b/3 global clinical trial of AMX0035 for the treatment of PSP, in December 2023. Data from an interim analysis of the ORION are expected in mid-2025. The Company is also advancing additional drug candidates developed by Amylyx for neurodegenerative diseases including AMX0114, a potent antisense oligonucleotide targeting inhibition of Calpain-2, a key contributor to the axonal (also known as Wallerian) degeneration pathway. The Company received clearance from Health Canada for its Clinical Trial Application for AMX0114 in people living with amyotrophic lateral sclerosis, or ALS, and plans to begin a Phase 1 multiple ascending dose, placebo-controlled trial called LUMINA in Canada by the end of 2024 or in early 2025.

In April 2024, the Company announced that it had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO and ALBRIOZA (AMX0035) for ALS and remove the product from the market based on topline results from the global Phase 3 PHOENIX trial, which did not meet its prespecified primary and secondary endpoints. Amylyx wound down the Open Label Extension as planned. The Company will continue to share learnings from PHOENIX to help inform future ALS research.

Risks and Uncertainties

The Company is subject to risks and uncertainties common to companies in the biotechnology industry, including, but not limited to, the outcome of preclinical studies and clinical trials, potential difficulties with or delays in timing with respect to regulatory approval processes, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, ability to secure additional capital to fund operations, and risks associated with the economic challenges caused by economic uncertainty in various global markets caused by geopolitical instability and conflict. The Company and its contractors may experience disruptions in supply of items that are essential for its research and development activities, including, for example, raw materials and bulk drug substances that the Company imports from Europe and Canada used in the manufacturing of AMX0035, avexitide and any additional or future product candidates.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Significant Accounting Policies

The Company's significant accounting policies are disclosed in the audited consolidated financial statements for the year ended December 31, 2023 and the notes thereto, which are included in the Company's most recent Annual Report on Form 10-K. Except as noted below, there have been no material changes to its significant accounting policies since the date of those consolidated financial statements.

Basis of Presentation and Consolidation

The accompanying condensed consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the U.S., or GAAP, and include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. In the opinion of the Company's management, all normal and recurring adjustments necessary for a fair presentation have been reflected. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the ASC, and Accounting Standards Update, or ASU, of the Financial Accounting Standards Board, or FASB.

Use of Estimates

The preparation of the condensed consolidated financial statements in conformity with GAAP requires the Company's management to make estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the financial statements and the reported amount of expenses during the reporting period. Actual results could differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies in developing the estimates and assumptions that are used in the preparation of the financial statements. Management must apply significant judgment in this process. Management's estimation process often may yield a range of potentially reasonable estimates and management must select an amount that falls within that range of reasonable estimates.

Business Combinations and Asset Acquisitions

The Company evaluates acquisitions of assets and other similar transactions to assess whether or not the transaction should be accounted for as a business combination or asset acquisition by first applying a screen test to determine if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets. If the screen test is met, the transaction is accounted for as an asset acquisition. If the screen test is not met, further determination is required as to whether or not the Company has acquired inputs and processes that have the ability to create outputs which would meet the requirements of a business. If determined to be an asset acquisition, the Company accounts for the transaction under ASC 805-50, which requires the acquiring entity in an asset acquisition to recognize assets acquired and liabilities assumed based on the cost to the acquiring entity on a relative fair value basis, which includes transaction costs in addition to consideration given. Goodwill is not recognized in an asset acquisition and any excess consideration transferred over the fair value of the net assets acquired is allocated to the identifiable assets based on relative fair values. In-process research and development, or IPR&D, projects with no alternative future use are recorded in R&D expense upon acquisition, and contingent consideration obligations incurred in connection with an asset acquisition are recorded when it is probable that they will occur and they can be reasonably estimated.

New Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvement to Income Tax Disclosures*, or ASU 2023-09, to enhance the transparency and decision usefulness of income tax disclosures. ASU 2023-09 is effective for annual periods beginning after December 15, 2024, on a prospective basis. Early adoption and retrospective application are permitted. The Company is currently evaluating the impact of this ASU on its consolidated financial statements and related disclosures.

In November 2023, the FASB issued ASU No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*, or ASU 2023-09, which requires public entities to disclose information about their reportable segments' significant expenses on an interim and annual basis. ASU 2023-07 is effective for the Company beginning the year ended December 31, 2024. The Company is currently evaluating the impact of this ASU on its consolidated financial statements and related disclosures.

3. PRODUCT REVENUE, NET

To date, the Company's only source of product revenue had been from sales of RELYVRIO, known as ALBRIOZA in Canada. Significant judgment was required in estimating gross-to-net, or GTN adjustments considering historical experience, payer channel mix (e.g., Medicare or Medicaid), current contract prices under applicable programs, unbilled claims and processing time lags and inventory levels in the distribution channel. In April 2024, the Company announced it had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ and remove the product from the market in the U.S. and Canada based on topline results from the Phase 3 PHOENIX trial. As a result, the Company did not generate significant revenue from product sales for the three months ended September 30, 2024 and the balance primarily represents gross-to-net adjustments reflecting actual rebate and return activity during the period. The following table reconciles gross product revenue to net product revenue (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Product revenue, gross	$ —	$ 111,864	$ 114,265	$ 304,822
GTN adjustments	416	(9,171)	(26,229)	(32,485)
Product revenue, net	$ 416	$ 102,693	$ 88,036	$ 272,337

The activity and ending reserve balance for GTN adjustments were as follows for the nine months ended September 30, 2024 and 2023 (in thousands):

	Chargebacks and Cash Discounts	Medicaid and Medicare Rebates	Other Rebates, Returns, Discounts and Adjustments	Total
Ending balance at December 31, 2023	$ 3,143	$ 4,946	$ 11,073	$ 19,162
Provision related to sales in the current year	4,983	3,504	20,916	29,403
Adjustments related to prior period sales	(1,696)	—	(1,478)	(3,174)
Credits and payments made	(6,430)	(8,087)	(11,551)	(26,068)
Ending balance at September 30, 2024	$ —	$ 363	$ 18,960	$ 19,323

	Chargebacks and Cash Discounts	Medicaid and Medicare Rebates	Other Rebates, Returns, Discounts and Adjustments	Total
Ending balance at December 31, 2022	$ 648	$ 1,992	$ 1,664	$ 4,304
Provision related to sales in the current year	14,175	7,872	10,954	33,001
Adjustments related to prior period sales	—	(236)	(280)	(516)
Credits and payments made	(11,004)	(6,806)	(8,930)	(26,740)
Ending balance at September 30, 2023	$ 3,819	$ 2,822	$ 3,408	$ 10,049

Included in the ending reserve balance for GTN adjustments are chargebacks resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to customers who directly purchase the product from the Company, discounts to customers for prompt payment and estimates for product returns. Chargebacks, discounts and returns are recorded as reductions of accounts receivable, net on the condensed consolidated balance sheets to the extent there are receivable balances to reduce. If there are net balance owed to customers, they are recorded as a component of accrued expenses on the condensed consolidated balance sheets. In addition, included in the ending reserve balance for GTN adjustments are Medicaid and Medicare rebates, other rebates for obligations under voluntary patient assistance programs, and accrued fees payable to customers. Medicaid and Medicare rebates, other rebates and fees are recorded as a component of accrued expenses on the condensed consolidated balance sheets.

4. MARKETABLE SECURITIES

The Company has classified all of its marketable securities at September 30, 2024 as "available-for-sale". The Company records available-for-sale securities at fair value, with the unrealized gains and losses included as a separate component of other accumulated comprehensive income (loss). There were no realized gains or losses recognized during the three and nine months ended September 30, 2024 and 2023.

The Company adjusts the cost of available-for-sale debt securities for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion are included in interest income. The cost of securities sold is based on the specific

identification method. The Company includes interest and dividends on securities classified as available-for-sale in interest income. Accrued interest receivable relating to the Company's available-for-sale securities is presented within prepaid expenses and other current assets in the accompanying condensed consolidated balance sheets, and amounted to less than $0.1 million and $0.5 million at September 30, 2024 and December 31, 2023, respectively.

As of September 30, 2024, there were no securities in an unrealized loss position for greater than 12 months. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost bases. The Company did not record an allowance for credit losses as of September 30, 2024.

Marketable securities, which are classified as available-for-sale, consisted of the following (in thousands):

Balance at September 30, 2024:	Amortized Cost Basis	Unrealized Gain	Unrealized Loss	Fair Value
Treasury bills	$ 162,148	$ 408	$ —	$ 162,556
Total marketable securities	$ 162,148	$ 408	$ —	$ 162,556

Balance at December 31, 2023:	Amortized Cost Basis	Unrealized Gain	Unrealized Loss	Fair Value
Treasury bills	$ 196,098	$ 67	$ —	$ 196,165
U.S. agency bonds	4,999	—	(3)	4,996
Total marketable securities	$ 201,097	$ 67	$ (3)	$ 201,161

5. INVENTORIES

Inventories consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Raw materials	$ —	$ 53,144
Work in process	—	18,945
Finished goods	—	11,191
Total inventories	$ —	$ 83,280

In April 2024, the Company announced it had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ and remove the product from the market in the U.S. and Canada based on topline results from the global Phase 3 PHOENIX trial. As a result, the Company recorded approximately $92.5 million of charges associated with the write-down of inventory for the nine months ended September 30, 2024. Inventory write-downs for the nine months ended September 30, 2023 were immaterial.

6. ACCRUED EXPENSES

Accrued expenses consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Accrued external research and development	$ 4,399	$ 12,625
Accrued benefits and incentive compensation	7,962	16,790
Accrued manufacturing	4,008	1,652
Accrued consulting and other professional fees	1,944	6,506
Accrued returns, rebates and co-pay assistance	18,834	16,063
Accrued royalties	—	3,111
Accrued loss on future purchase commitments	12,743	—
Other accrued expenses	566	977
Total accrued expenses	$ 50,456	$ 57,724

7. FAIR VALUE MEASUREMENTS

The following tables present information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicates the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	September 30, 2024			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 38,813	$ —	$ —	$ 38,813
Marketable securities:				
Treasury bills	162,556	—	—	162,556
Total marketable securities	162,556	—	—	162,556
Restricted cash equivalents	1,441	—	—	1,441
Total financial assets	$ 202,810	$ —	$ —	$ 202,810

	December 31, 2023			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 76,710	$ —	$ —	$ 76,710
Marketable securities:				
Treasury bills	196,165	—	—	196,165
U.S. agency bonds	—	4,996	—	4,996
Total marketable securities	196,165	4,996	—	201,161
Restricted cash equivalents	719	—	—	719
Total financial assets	$ 273,594	$ 4,996	$ —	$ 278,590

The Company classifies its money market funds and treasury bills as Level 1 assets under the fair value hierarchy, as these assets have been valued using quoted market prices for identical assets in active markets without any valuation adjustment. The Company classifies its U.S. agency bonds as Level 2 assets under the fair value hierarchy, as these assets have been valued using information obtained through a third-party pricing service at each balance sheet date, using observable market inputs for similar assets that may include trade information, broker or dealer quotes, bids, offers, or a combination of these data sources.

8. STOCK OPTION AND GRANT PLAN

Stock Incentive Plan

General Option Information

A summary of option activity for the nine months ended September 30, 2024, is as follows:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2023	9,823,248	$ 18.39	7.9	$ 27,639
Granted	3,056,911	$ 8.56		
Exercised	(146,398)	$ 1.55		$ 780
Cancelled or forfeited	(4,390,010)	$ 19.47		
Outstanding at September 30, 2024	8,343,751	$ 14.52	7.7	$ 2,576
Options exercisable as of September 30, 2024	3,864,567	$ 15.57	6.4	$ 579
Options unvested as of September 30, 2024	4,479,184	$ 13.59	8.8	$ 1,996
Weighted average grant-date fair value of options granted during the period	$ 5.61			

The aggregate intrinsic value of options exercised during the nine months ended September 30, 2024 and 2023 was $0.8 million and $18.4 million, respectively.

The total fair value of stock options vested during the nine months ended September 30, 2024 and 2023 was $28.2 million and $24.8 million, respectively.

Restricted Stock Unit Activity

A summary of restricted stock unit activity for the nine months ended September 30, 2024, is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value
Nonvested as of December 31, 2023	1,112,542	$ 24.80
Granted	2,826,628	$ 8.80
Vested	(691,678)	$ 10.69
Forfeited	(979,758)	$ 20.39
Nonvested as of September 30, 2024	2,267,734	$ 11.06

Summary of Stock-Based Compensation Expense

Stock-based compensation expense for the three and nine months ended September 30, 2024 and 2023, is as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development	$ 1,834	$ 2,664	$ 6,995	$ 7,090
Selling, general and administrative	4,969	7,512	19,302	20,537
Total stock-based compensation expense	$ 6,803	$ 10,176	$ 26,297	$ 27,627

The following table summarizes unrecognized stock-based compensation expense as of September 30, 2024, by type of awards (in thousands), and the weighted-average period over which that expense is expected to be recognized (in years). The total unrecognized stock-based compensation expense will be adjusted for actual forfeitures as they occur.

	September 30, 2024	
	Unrecognized Expense	Weighted-average Recognition Period
Stock options	$ 37,484	2.31
Restricted stock units	$ 20,498	2.70

9. NET (LOSS) INCOME PER SHARE

Net (Loss) Income per Share

Basic earnings per share is computed by dividing net income (loss) by the weighted average number of common shares outstanding during the period. Diluted earnings per share is calculated based on the combined weighted average number of common shares and potentially dilutive shares, which include the assumed exercise of employee stock options and unvested restricted stock units. In computing diluted earnings per share, the Company utilizes the treasury stock method.

A summary of the numerator and denominators used in the computation of earnings per share follows (in thousands, except share and per share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Numerator:				
Net (loss) income	$ (72,704)	$ 20,893	$ (264,197)	$ 44,540
Denominator:				
Weighted-average shares used to compute basic net (loss) income per share	68,091,446	67,414,669	67,990,613	67,124,407
Dilutive effect of employee stock options and restricted stock units	—	2,333,878	—	3,019,252
Weighted-average shares used to compute diluted net (loss) income per share	68,091,446	69,748,547	67,990,613	70,143,659
Net (loss) income per share				
Basic	$ (1.07)	$ 0.31	$ (3.89)	$ 0.66
Diluted	$ (1.07)	$ 0.30	$ (3.89)	$ 0.63

Because the Company reported a net loss for the three and nine months ended September 30, 2024, basic and diluted net loss per share attributable to common stockholders were the same. All stock options and restricted stock units were excluded from the computation of diluted weighted-average shares outstanding because such securities would have an antidilutive impact for the three and nine months ended September 30, 2024. The following stock options and restricted stock units outstanding at each period end have been excluded from the calculation of diluted net income (loss) per share because their inclusion would have been antidilutive:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Options to purchase common stock	8,343,751	5,971,476	8,343,751	5,937,376
Restricted stock units	2,267,734	626,086	2,267,734	510,173
Total excluded common stock equivalents	10,611,485	6,597,562	10,611,485	6,447,549

10. ACQUISITIONS

On July 9, 2024, the Company completed the acquisition of substantially all the assets and interests in the development, manufacture and commercialization of avexitide from Eiger BioPharmaceuticals, Inc., or Eiger, for $35.1 million, or the Eiger Acquisition. The Eiger Acquisition includes the acquisition of all of Eiger's owned and co-owned patents and applications directed to avexitide, as well as the assumption of Eiger's licenses to patents and applications directed to avexitide and owned and co-owned by other entities, and the samples, retains, raw materials and active pharmaceutical ingredients in Eiger's possession and control.

The transaction was accounted for as an asset acquisition as the acquired assets did not meet the definition of a business. The Company did not acquire any outputs and there was not an acquired substantive process in place to create outputs. The total purchase consideration of $36.2 million was composed of cash paid at closing of $35.1 million and direct transaction costs of $1.1 million.

The fair value was allocated to IPR&D assets with no alternative future use for these assets at the closing of the acquisition. As a result, the Company recorded a charge of $36.2 million related to acquired in-process research and development expense on the condensed consolidated statements of operations during the three and nine months ended September 30, 2024.

As part of the transaction, the Company assumed certain contractual obligations from Eiger, including royalty obligations between 4% and 7% on future sales owed to certain academic institutions and individuals. The Company will recognize these royalty payments related to avexitide in the period in which the achievement of the underlying milestones becomes probable. There were no other contingent obligations or assumed liabilities from the acquisition as of September 30, 2024.

11. COMMITMENTS AND CONTINGENCIES

Letter of Credit

Restricted cash equivalents consist of $0.9 million of cash serving as collateral for a letter of credit issued for the Company's office spaces, and $0.5 million as collateral for a corporate credit card program. As of September 30, 2024 and December 31, 2023, the Company's restricted cash equivalents balance was $1.4 million and $0.7 million, respectively, on its condensed consolidated balance sheets.

Legal Proceedings

On February 9, 2024, a putative class action lawsuit was filed in the U.S. District Court for the Southern District of New York against the Company and certain of its current and former officers (*Shih v. Amylyx Pharmaceuticals, Inc., et al.*, Case Number 1:24-CV-00988, or the Shih Complaint). Plaintiff filed an amended complaint on June 24, 2024. The Shih Complaint asserts a claim against all defendants for alleged violations of Section 10(b) of the Exchange Act and Rule 10b-5 promulgated thereunder and a claim under Section 20(a) against certain current and former officers as alleged controlling persons. The Shih Complaint alleges that defendants made materially false and misleading statements related to the commercial results and prospects for RELYVRIO. The Shih Complaint seeks unspecified damages, interest, costs and attorneys' fees, and other unspecified relief that the court deems appropriate.

On August 12, 2024, the case was transferred from the U.S. District Court for the Southern District of New York to the U.S. District Court for the District of Massachusetts and assigned docket number 1:24-CV-12068. Following the transfer, on September 6, 2024, defendants moved to dismiss the Shih Complaint. Plaintiff filed his opposition to Defendants' motion to dismiss on October 21, 2024 and defendants' response is due on or before November 20, 2024. The Company intends to defend against the Shih Complaint vigorously. At this time, an estimate of the impact, if any, of the claims made in the Shih Complaint cannot be made.

Royalty Payments

Between August 2016 and February 2019, the Company entered into grant agreements with the ALS Association, ALS Finding a Cure Foundation, Alzheimer's Drug Discovery Foundation, Alzheimer's Association and Cure Alzheimer's Fund, or Grantors. Under the terms of the respective grant agreements among the Company, Alzheimer's Drug Discovery Foundation, the Alzheimer's Association, and Cure Alzheimer's Fund, the Company will make royalty payments up to the maximum amount of $15.0 million to each Grantor (or $45.0 million in aggregate). As the conditions that would trigger royalty payments under the agreements have not occurred, no amounts have been recorded in the condensed consolidated financial statements.

As disclosed in Note 10 *Acquisitions,* the Company assumed royalty obligations from Eiger related to the acquisition of avexitide. As the conditions that would trigger royalty payments under the agreements have not occurred, no amounts have been recorded in the condensed consolidated financial statements.

Purchase Commitments

The Company enters into agreements in the normal course of business with contract manufacturing organizations for raw material purchases and manufacturing services. As of September 30, 2024, the Company had approximately $27.2 million of remaining obligations under these agreements, which are expected to be paid through 2025.

The Company recognized a loss on purchase commitments of $0.8 million and $26.1 million for the three and nine months ended September 30, 2024, respectively, which was recorded to cost of sales on the condensed consolidated statement of operations and as part of accrued expenses on the condensed consolidated balance sheet. The purchase commitment loss is based on an estimate of future commitments related to supply agreements with third party vendors for which the Company does not expect to have related sales.

Facility Leases

On September 12, 2024, the Company entered into a new office lease in Cambridge, Massachusetts for office space for its headquarters facility. The lease provides office space of approximately 15,267 square feet and for base monthly rent payments beginning at $0.1 million that increase annually by approximately 2.5% over the term of five years from the date of occupancy. In addition to base rent, the Company has agreed to reimburse the landlord for certain operating expenses under the terms of the lease. The lease commencement date is expected to be June 1, 2025 when the premises are expected to be available for occupancy and, therefore, as the office lease has not commenced, the related operating lease right-of-use assets and liabilities are not recorded in the Company's condensed consolidated balance sheet as of September 30, 2024.

12. RESTRUCTURING

In April 2024, the Company announced a restructuring plan designed to focus the Company's resources on key clinical and preclinical programs, or the Restructuring Plan. The Restructuring Plan included a reduction in force which reduced the Company's workforce by approximately 70% and a decrease in external financial commitments outside of its priority areas. The Company substantially completed the Restructuring Plan in the second quarter of 2024.

Restructuring expense consists primarily of employee severance and termination benefits, contract termination costs, impairment of long-lived assets and other costs. Liabilities for costs associated with a restructuring activity are recognized when the liability is incurred and are measured at fair value. According to ASC 420, *Exit or Disposal Cost Obligations*, one-time employee severance and termination benefits are expensed at the date the entity notifies the employee of the plan, unless the employee must provide future service, in which case the benefits are expensed in the period when the service ends. One-time termination benefits primarily include severance, continuation of health insurance coverage, and other benefits such as outplacement support services for a specified period of time.

In connection with the Restructuring Plan, the Company performed an impairment evaluation of its long-lived assets resulting in an impairment charge of $0.9 million during the nine months ended September 30, 2024 related to the impairment of capitalized internal-use software.

Restructuring expenses for the three months ended September 30, 2024 were as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Severance and employee benefit costs	$ —	$ —	$ 21,812	$ —
Contract termination costs, impairment of assets and other costs	—	—	1,039	—
Total	$ —	$ —	$ 22,851	$ —

All accrued costs related to this restructuring activity were paid as of September 30, 2024.

13. SUBSEQUENT EVENTS

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The Company has evaluated all subsequent events after September 30, 2024 and through the date of this filing, and there were no material subsequent events requiring disclosure, except as described below:

On October 2, 2024, a derivative complaint was filed in the U.S. District Court for the District of Massachusetts against certain current and former director and officer defendants, naming the Company as nominal defendant (*Jones v. Cohen, et al.*, 1:24-CV-12527, or the Derivative Complaint). The substantive allegations mirror those of the Shih Complaint but also include claims for alleged violations of Section 14(a) of the Exchange Act, breach of fiduciary duty, insider trading, and unjust enrichment. The Derivative Complaint seeks unspecified damages to be awarded to the Company along with interest, costs, and attorneys' fees, restitution, and unspecified corporate governance and internal procedural reforms and improvements. On October 31, 2024, the Court entered an order staying the action until the earlier of the dismissal of the Shih Complaint with prejudice, including the exhaustion of all appeals, or defendants file an answer to the Shih Complaint. The Company intends to defend against the Derivative Complaint vigorously. At this time, an estimate of the impact, if any, of the claims made in the Derivative Complaint cannot be made.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following information should be read in conjunction with the condensed consolidated financial information and the notes thereto appearing elsewhere in this Quarterly Report.

This discussion and other parts of this Quarterly Report contain forward-looking statements that involve risks and uncertainties, such as statements of our plans, objectives, expectations and intentions. As a result of many factors, including those risk factors set forth in our most recent Annual Report on Form 10-K, or 2023 Annual Report, and in our subsequent Quarterly Reports, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a biotechnology company committed to the discovery and development of new treatment options for communities with high unmet needs, including people living with serious and fatal diseases. We have preclinical and clinical development programs underway in neurodegenerative diseases and endocrine conditions. Our pipeline of programs includes avexitide for the treatment of hyperinsulinemic hypoglycemia, AMX0035 for the treatment of Wolfram syndrome, AMX0035 for the treatment of progressive supranuclear palsy, or PSP, and AMX0114, our antisense oligonucleotide targeting calpain-2 for the treatment of amyotrophic lateral sclerosis, or ALS.

Avexitide, an investigational, first-in-class glucagon-like peptide-1, or GLP-1, receptor antagonist, has been evaluated in five clinical trials for post-bariatric hypoglycemia, or PBH, and has also been studied in congenital hyperinsulinism, or HI, two indications characterized by hyperinsulinemic hypoglycemia. The U.S. Food and Drug Administration, or FDA, has granted avexitide breakthrough therapy designation for both indications, rare pediatric disease designation in congenital HI, and orphan drug designation for the treatment of hyperinsulinemic hypoglycemia (which includes PBH and congenital HI). Avexitide is designed to bind to the GLP-1 receptor on pancreatic islet beta cells and block the effect of GLP-1 to mitigate hypoglycemia by decreasing insulin secretion and stabilizing glucose levels. In PBH, an indication with no approved treatment options impacting approximately 160,000 people in the U.S., excessive GLP-1 can lead to the hypersecretion of insulin and subsequent severe hypoglycemia events, including autonomic and neuroglycopenic symptoms if left unaddressed. Avexitide was generally well tolerated, with a favorable safety profile replicated across five clinical trials in people with PBH.

In previous Phase 2 and Phase 2b studies in PBH, avexitide showed statistically significant reductions in hypoglycemia events characterized by low blood glucose, including severe hypoglycemia events with altered mental and/or physical function requiring assistance. FDA guidance for industry in diabetes combined with initial FDA feedback specific to the pivotal Phase 3 program of avexitide for PBH suggest that reduction in hypoglycemia events could be an endpoint to support approval following positive results from a pivotal Phase 3 clinical trial. We expect to begin a Phase 3 program for avexitide in PBH in the first quarter of 2025 with topline data anticipated in 2026. We are actively engaging in discussions with the broader congenital HI community to develop a path forward.

AMX0035 is a specially formulated oral fixed-dose combination of sodium phenylbutyrate, or PB, and taurursodiol, or TURSO, developed to target a broad range of neurodegenerative diseases. AMX0035 was developed by Amylyx and is designed to mitigate neurodegeneration by simultaneously targeting endoplasmic reticulum, or ER, stress and mitochondrial dysfunction, two connected central pathways that lead to cell death and neurodegeneration. We have shown in multiple models that AMX0035 can keep neurons alive under a variety of different conditions and stresses. These conditions include *in vitro* models of neurodegeneration, ER stress, mitochondrial dysfunction, oxidative stress and various other disease-specific models, as well as *in vivo* models of ALS, Wolfram syndrome, Alzheimer's disease, or AD, and multiple sclerosis, or MS. Trials of AMX0035 in AD and ALS have shown the ability to reduce markers associated with neurodegenerative diseases in clinical trials, including a reduction of tau, a key protein aggregate shared across several neurodegenerative diseases, and YKL-40, a marker of neuroinflammation, respectively.

The safety profile of AMX0035 has been well established in clinical trials and post-marketing surveillance. Thousands of adults have been treated with AMX0035 whether through clinical trials across several neurodegenerative conditions and in post-approval settings. AMX0035 has been generally well-tolerated and the most common adverse reactions were gastrointestinal side effects (e.g. diarrhea, nausea, constipation, decreased appetite, abdominal pain, flatulence), occurring more often in the first 3 weeks of treatment and were generally nonserious. The benefit-risk balance of AMX0035 remains favorable for ongoing clinical development programs.

We are investigating AMX0035 in neurodegenerative diseases and endocrine conditions where ER stress and mitochondrial dysfunction are implicated, including PSP and Wolfram syndrome.

Wolfram syndrome is a rare, progressive monogenic disease caused by mutations in the WFS1 gene that cause ER stress and mitochondrial dysfunction, leading to multi-organ cell dysfunction and death. We believe Wolfram syndrome impacts approximately 3,000 people in the U.S. Researchers from the Washington University School of Medicine in St. Louis, in collaboration with Amylyx, published preclinical data exploring the potential of AMX0035 as a novel therapeutic approach for Wolfram syndrome in the peer-reviewed *Journal of Clinical Investigation Insight*. These data showed that AMX0035 had the ability to dampen the impact of ER stress and mitochondrial dysfunction across patient-derived beta cells and neurons impacted by WFS1 mutations, and thereby potentially stabilizing and in some cases improving cellular functioning and viability. Amylyx announced that the FDA granted orphan drug designation to AMX0035 for the treatment of Wolfram syndrome in November 2020.

On October 17, 2024, we announced positive topline data from the HELIOS trial, an ongoing, open-label Phase 2 study designed to evaluate if AMX0035 slows progression of diabetic, visual, and other measures in people living with Wolfram syndrome and to evaluate safety and tolerability. HELIOS showed improvement in pancreatic function, as measured by C-peptide response after 24 weeks of treatment with AMX0035, the study's primary efficacy endpoint, in contrast to the expected decrease in pancreatic function with disease progression. Similar overall improvements or stabilization were observed across all secondary endpoints, including hemoglobin A1c (HbA1c), time in target glucose range assessed by continuous glucose monitoring, and visual acuity. Patient- and physician-reported global impressions of change showed disease stability or improvement in all participants, meeting prespecified responder criteria. In addition, longer-term data for all participants who completed Week 36 (n=10) and Week 48 (n=6) assessments showed sustained improvement over time. The analysis performed includes Week 24 data for all 12 participants and data for all participants who completed their Week 36 (n=10) and Week 48 (n=6) assessments as of the data cutoff.

HELIOS showed improvements in its primary endpoint of C-peptide response with a change from baseline to Week 24 at 120 minutes of +3.8 minutes*ng/mL (min*ng/mL) [standard error (SE): 19.3] in the Intent to Treat group (N=12) and +20.2 min*ng/mL [SE: 11.2] in the Per Protocol group (N=11). In addition, as outlined in the table below, participants receiving AMX0035 had improved glycemic control, as measured by markers of glucose metabolism; improved visual acuity in some participants, as measured by the Snellen chart; and improvement or stabilization of the disease, as measured by the Clinician Reported Global Impression of Change (CGIC) and Patient Reported Global Impression of Change (PGIC).

	Week 24 ITT (N-12)	**Week 24 Per Protocol† (N=11)**	**Week 36 (n=10)**	**Week 48 (n=6)**
C-Peptide Response (min*ng/mL) *mean change in AUC from baseline over* 120 minutes††	+3.8 (SE: 19.3)	+20.2 (SE: 11.2)	+30.7 (SE: 9.7)	+36.7 (SE: 19.6)
Hemoglobin A1c (%) *change from baseline*	-0.09 (SE: 0.14)	-0.16 (SE: 0.13)	-0.35 (SE: 0.18)	-0.30 (SE: 0.31)
Absolute Time in Target Glucose Range (%) *change from baseline*	+5.2 (SE: 3.6)	+5.7 (SE: 3.9)	+12.3 (SE: 4.0)	+5.8 (SE: 8.9)
Mean Exogenous Insulin Dose (units/kg/2 weeks) *change from baseline*	-0.01	-0.01	0.01	0.02
Visual Acuity (LogMAR) *change from baseline*	-0.04 (SE: 0.06)	-0.04 (SE: 0.06)	Not Collected at this Time Point	-0.11 (SE: 0.12)
Clinician Report Global Impression of Change (CGIC) *% meeting responder criteria†††*	100%	100%	100%	100%
Patient Reported Global Impression of Change (PGIC) % meeting responder criteria†††	100%	100%	100%	100%

† Upon genetic review, one participant did not meet the inclusion/exclusion criteria for HELIOS. This participant was found to have an autosomal recessive mutation confirmed to be pathogenic on just one of the two alleles and variant of uncertain significance on the other allele. This participant was within normal range for C-peptide, glycemic measures, and vision suggesting lack of typical Wolfram syndrome phenotype. Data presented with and without this participant who reached Week 24 (Intent to Treat and Per Protocol, respectively).
†† In non-diabetic individuals, C-peptide peaks after a meal at approximately ~30 minutes; in Wolfram syndrome, peak is slower but generally was at or before 120 minutes in HELIOS. Area under the curve (AUC) over 120 minutes after meal challenge reflects beta cell response to a meal. Amylyx is currently planning to focus on 120-minute AUC as the C-peptide measure for future studies.
††† HELIOS defines a "responder" on both the CGIC and PGIC as no change or improvement given the progressive nature of Wolfram syndrome.

The primary efficacy endpoint of the trial measures change from baseline in C-peptide, an established, objective laboratory measure of pancreatic beta cell function and glycemic control, was assessed using a Mixed Meal Tolerance Test at Week 24. Secondary and exploratory outcomes include the measurement of other diabetic responses and other domains affected by the disease. The safety profile of AMX0035 was consistent with that observed in other populations in which the drug has been extensively studied. We are engaging with stakeholders, including the FDA, and planning for a single Phase 3 clinical trial and will share additional details on the clinical trial design once finalized.

We are pursuing AMX0035 for the treatment of PSP based on AMX002's mechanism, preclinical data, and the tau reduction seen in the Phase 2 PEGASUS trial of AMX0035 in AD. These data were published in the peer-reviewed medical journal *Alzheimer's & Dementia: Translational Research & Clinical Interventions*, a journal of the Alzheimer's Association, in August 2024. PSP is a rare neurodegenerative disorder that affects body movements, walking, balance and eye movement and is characterized by widespread neurodegeneration associated with tau protein deposition in subcortical regions of the brain. Based on *in vitro, in vivo* and clinical data, AMX0035 may influence tau pathology, a key hallmark in people living with PSP. Genetic evidence indicates that a risk factor for PSP may be the unfolded protein response activated in disease-affected regions of the brain. PB has been shown to upregulate and recruit chaperone proteins, stabilize protein folding, and reduce ER stress and the unfolded protein response, while TURSO has been shown to inhibit tau phosphorylation induced by metabolic stress. In addition to preclinical data on PB and TURSO individually, PB and TURSO combined have shown a synergistic effect in targeting the hallmark pathways of neurodegeneration to simultaneously prevent or slow neuronal cell death. There are currently no approved therapies for the treatment of PSP, and the disease is reported to affect seven in 100,000 people worldwide.

The ORION trial, a Phase 2b/3 global trial of AMX0035 for the treatment of PSP, remains ongoing. The primary efficacy endpoint of the ORION trial evaluates disease progression as measured by the rate of change on the 28-item Progressive Supranuclear Palsy Rating Scale (PSPRS), total score at Week 52, an established and validated endpoint in PSP clinical trials. Secondary efficacy endpoints are disease progression as measured by a modified 10-item PSPRS score and motor aspects of activities of daily life as measured by the Movement Disorder Society-Unified Parkinson's Disease Rating Scale Part 2 (MDS-UPDRS Part II). Exploratory outcomes include changes in activities of daily living, cognitive function, quality of life, overall survival, brain regional volumes, fluid biomarkers of neuronal injury/inflammation, and caregiver burden. Safety and tolerability will be evaluated by assessing the frequency of treatment emergent adverse events (TEAEs) and serious adverse events (SAEs). Data from an interim analysis of the ORION study of AMX0035 in PSP are expected in mid-2025.

Additionally, we remain steadfast in our commitment to the ALS community. ALS is a disorder characterized by motor neuron degeneration in the motor cortex and spinal cord that leads to progressive muscle weakness. Unlike most other cells in the body that regularly die and are replaced as part of healthy function, mature central nervous system (CNS) neurons are normally resistant to cell death and generally cannot regenerate. There are approximately 30,000 people living with ALS in the U.S. ALS is a disease that affects people regardless of background, and there are approximately 200,000 people living worldwide with ALS.

To that end, we have completed the IND-enabling studies of AMX0114, a potent antisense oligonucleotide targeting inhibition of Calpain-2, a key contributor to the axonal (also known as Wallerian) degeneration pathway. Axonal degeneration has been recognized as an important early contributor to the clinical presentation and pathogenesis of ALS and other neurodegenerative diseases. Calpain-2 is considered an essential protein in the process of axonal degeneration and has been repeatedly linked to neurofilament biology in published studies. Preclinical studies completed to date have shown that AMX0114 can achieve potent, dose-dependent, and durable knockdown of CAPN2 mRNA expression and Calpain-2 protein levels in human motor neurons. Moreover, in preclinical efficacy studies, treatment with AMX0114 reduced extracellular neurofilament light chain levels following neurotoxic insult in induced pluripotent stem cell (iPSC)-derived human motor neurons, and improved survival of iPSC-derived human motor neurons harboring ALS-linked, pathogenic TDP-43 mutations. Neurofilament is a broadly researched biomarker in ALS.

In ALS, we received clearance from Health Canada for our Clinical Trial Application for AMX0114 in people living with ALS. We plan to begin the Phase 1 multiple ascending dose, placebo-controlled trial called LUMINA in Canada by the end of 2024 or in early 2025. We will evaluate safety and the biological activity in approximately 48 adults living with ALS and evaluate four dose levels, starting with 12.5 mg. We presented our plans for the study at the Northeast ALS Consortium Annual Meeting last month.

We also submitted an Investigational New Drug application to the FDA for AMX0114. The FDA restricted dosing to an amount that is lower than the Company's proposed starting dose of 12.5 mg and has requested additional information, which resulted in a clinical hold. Toxicology data from studies showed a greater than 10X safety margin at the starting dose of 12.5 mg based on the no observed adverse effect level (NOAEL) determined by independent toxicology firms. We are working to address FDA comments. We believe the trial can be completed outside of the U.S if needed. We expect early cohort data from LUMINA in 2025.

On April 4, 2024, we announced we had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ (sodium phenylbutyrate and taurursodiol [also known as

ursodoxicoltaurine]; also known as AMX0035) for the treatment of ALS and remove the product from the market in the U.S. and Canada, or the "RELYVRIO®/ALBRIOZA™ Discontinuation. This decision was informed by topline results from the global Phase 3 PHOENIX trial, which failed to meet its prespecified primary and secondary endpoints, engagement with regulatory authorities, and discussions with the ALS community. As of April 4, 2024, RELYVRIO/ALBRIOZA was no longer available for new patients. Patients who were currently on therapy in the U.S. and Canada who, in consultation with their physician, wished to stay on treatment had the option to be transitioned to a free drug program. Patients and their physicians were informed that final shipments of free drug were shipped to allow treatment through early 2025. The NDA is now on the Discontinued Drug Product List of the Orange Book. We will continue to collect available data on survival and to share learnings from PHOENIX to help inform future ALS research. We wound down the Open Label Extension as planned.

Since inception, we have devoted a substantial amount of our efforts to research and development and pre-commercialization and commercialization activities, including recruiting management and technical staff, raising capital, producing materials for preclinical studies and clinical trials, and building infrastructure to support such activities. As of September 30, 2024, we have funded our operations primarily through public offerings of our common stock, private sales of preferred stock, convertible notes, and through revenue from sales of RELYVRIO and ALBRIOZA in the U.S. and Canada, respectively.

As of September 30, 2024, we had cash, cash equivalents and marketable securities of $234.4 million. In April 2024, we announced a restructuring to focus our financial resources on upcoming clinical milestones. Under the restructuring, we reduced our workforce by approximately 70% and decreased external financial commitments outside of our priority areas. We believe that with these changes, our existing cash, cash equivalents and marketable securities as of September 30, 2024, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least one year from the issuance of this Quarterly Report. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See "*Liquidity and Capital Resources*" below.

We will continue to incur significant expenses as we advance avexitide, AMX0035, AMX0114 and advance or acquire any future product candidates through preclinical and clinical development, setup and initiate additional trials, hire additional clinical, scientific, management and administrative personnel, seek regulatory approval and pursue commercialization of any approved product candidates. We may also incur expenses related to business development activities, such as in-licensing of product candidates.

We expect to finance our near-term operations through our existing cash, cash equivalents and marketable securities and if needed, the sale of equity, debt financings or other capital sources, including potential collaborations with other companies, royalty financings, or other strategic transactions. Our inability to raise capital or secure other funding as and when needed could have a negative impact on our financial condition and ability to pursue our business strategies. There can be no assurances that our current operating plan will be achieved or that additional funding, if required, will be available on terms acceptable to us, or at all.

Components of Our Results of Operations

Product Revenue, Net

Product revenue, net recognized during the three and nine months ended September 30, 2024 and 2023 primarily relates to units of ALBRIOZA and RELYVRIO sold in Canada and the U.S., respectively. In April 2024, we announced we had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ and remove the product from the market in the U.S. and Canada based on topline results from the global Phase 3 PHOENIX trial. As a result, we will not generate revenue from the sale of RELYVRIO®/ALBRIOZA™ in future periods.

Operating Expenses

Cost of Sales

Cost of sales consists primarily of costs associated with the manufacturing of RELYVRIO, ALBRIOZA, certain period costs and losses on purchase commitments with contract manufacturing organizations. Following our announcement of a process to discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ and remove the product from the market in the U.S. and Canada, we will not report product cost of sales in future periods.

Acquired In-process Research and Development Expenses

Acquired in-process research and development, or IPR&D, expenses include consideration for the purchase of substantially all the assets and interests in the development, manufacture and commercialization of avexitide from Eiger.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the research and development of AMX0035, avexitide, AMX0114 and other potential future product candidates. We expense research and development costs as incurred. These expenses include:

- expenses incurred under agreements with CROs, contract manufacturing organizations, or CMOs, as well as investigative sites and consultants that conduct our clinical trials, preclinical studies and other scientific development services;
- manufacturing scale-up expenses and the cost of acquiring and manufacturing drug product for our preclinical studies and clinical trials, including manufacturing registration and validation batches, as well as pre-commercial manufacturing activities;
- expenses to acquire technologies to be used in research and development;
- employee-related expenses, including salaries, payroll taxes, related benefits and stock-based compensation expense for employees engaged in research and development functions; and
- costs related to compliance with quality and regulatory requirements.

Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered, or the services rendered.

Certain of our indirect research and development expenses are not tracked on an indication-by-indication basis for AMX0035. We do not allocate employee costs and facilities, including depreciation or other indirect costs, to specific indications because these costs are deployed across multiple indications and, as such, are not separately classified. We use internal resources to oversee the research and discovery as well as to manage our preclinical development, process development, manufacturing and clinical development activities. These employees work across multiple indications and, therefore, we do not track their costs by indication.

Research and development activities are central to our business model. Product candidates such as AMX0035 in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, such as AMX0114, primarily due to the increased size and duration of later-stage clinical trials and related product manufacturing expenses. We expect that our research and development expenses will continue to increase in connection with our planned clinical development activities in the near term and in the future. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the clinical development of AMX0035, avexitide and any future product candidates. Our clinical development costs may vary significantly based on factors such as:

- per patient trial costs;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the number of patients that participate in the trials;
- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up periods;
- the cost and timing of manufacturing our current or future product candidates;

•the phase of development of our current or future product candidates;

•the efficacy and safety profile from clinical trials and preclinical studies of our current or future product candidates; and

•the number of product candidates we are developing.

The successful development and commercialization of AMX0035, avexitide and any other current or future product candidates is highly uncertain, due to the numerous risks and uncertainties associated with product development and commercialization, including the following:

•the timing and progress of preclinical and clinical development activities;

•the number and scope of preclinical and clinical trials for separate indications we decide to pursue;

•raising additional funds, if necessary;

•the progress of the development efforts of parties with whom we may enter into collaboration arrangements;

•our ability to maintain our current development activities and to establish new ones;

•our ability to establish new licensing or collaboration arrangements;

•the successful initiation and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any other comparable foreign regulatory authority;

•the availability of drug substance and drug product for use in production of AMX0035 or other product candidates;

•establishing and maintaining agreements with third-party manufacturers for clinical supply for our clinical trials;

•our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the U.S. and internationally;

•our ability to protect our rights in our intellectual property portfolio;

•obtaining and maintaining third-party insurance coverage and adequate reimbursement for any approved products;

•the acceptance of our products and product candidates, if approved, by patients, the medical community and third-party payors;

•competition with other products; and

•a continued acceptable safety profile of our therapies in pre-approval market access programs or in commercial access following approval.

A change in the outcome of any of these variables with respect to the development of AMX0035, avexitide or any other current or future product candidates could have a significant impact on the cost and timing associated with the development of our product candidates. We may never succeed in obtaining or maintaining, as applicable, regulatory approval for AMX0035, avexitide or any other current or future product candidates.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of salaries and related costs for personnel in executive, finance, sales, marketing, as well as administrative functions. Selling, general and administrative expenses also include legal fees relating to patent and corporate matters; professional fees for accounting, auditing, tax, compliance with Section 404 of the Sarbanes-Oxley Act, or Section 404, and administrative consulting services; corporate insurance costs; administrative travel expenses; sales and marketing expenses; information technology; charitable donations to independent charitable foundations; facility-related and other operating costs. In April 2024, we announced the Restructuring Plan designed to focus our resources on key clinical and preclinical programs. The restructuring included a reduction in force which is expected to reduce our workforce by approximately 70% and a decrease in external financial commitments outside our priority areas. As a result, we anticipate that our selling, general and administrative expenses will decrease in 2024 as compared to 2023.

Restructuring Expenses

Restructuring expense consists primarily of employee severance and termination benefits, contract termination costs, impairment of long-lived assets and other costs. We records cost and liabilities associated with exit and disposal activities in accordance with FASB ASC Topic 420, Exit or Disposal Cost Obligations. Such costs are based on estimates of fair value in the period liabilities are incurred. We evaluate and adjust these costs as appropriate for changes in circumstances as additional information becomes available.

Income Taxes

Income taxes are determined using the asset and liability approach. Deferred tax assets and liabilities represent future tax consequences of temporary differences between the financial statement carrying amounts and the tax basis of assets and liabilities and for tax attribute carryforwards using enacted tax rates expected to be in effect in the years in which the differences reverse. Realization of our deferred tax assets is dependent upon the generation of future taxable income, the amount and timing of which are uncertain. Valuation allowances are provided, if, based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. We continue to maintain a full valuation allowance against all of our deferred tax assets based on management's evaluation of all available evidence, including our history of incurring significant losses from operations. As a result, we don't expect to incur material income taxes for the foreseeable future.

Results of Operations

Comparison of the three months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the periods presented (in thousands):

	Three Months Ended September 30,			
	2024	2023	$ Change	% Change
Product revenue, net	$ 416	$ 102,693	$ (102,277)	(100)%
Operating expenses:				
Cost of sales	—	5,218	(5,218)	(100)%
Cost of sales - inventory impairment and loss on firm purchase commitments	809	—	809	*NM
Acquired in-process research and development	36,203	—	36,203	*NM
Research and development	21,237	30,037	(8,800)	(29)%
Selling, general and administrative	17,828	48,718	(30,890)	(63)%
Total operating expenses	76,077	83,973	(7,896)	(9)%
(Loss) income from operations	(75,661)	18,720	(94,381)	(504)%
Other income, net:				
Interest income	3,098	4,179	(1,081)	(26)%
Other expense, net	(141)	(488)	347	(71)%
Total other income, net	2,957	3,691	(734)	(20)%
(Loss) income before income taxes	(72,704)	22,411	(95,115)	(424)%
Provision for income taxes	—	1,518	(1,518)	(100)%
Net (loss) income	$ (72,704)	$ 20,893	$ (93,597)	(448)%

Product Revenue, Net and Cost of Sales

As a result of the RELYVRIO®/ALBRIOZA™ Discontinuation, we did not generate significant revenue from product sales for the three months ended September 30, 2024 and the balance primarily represents gross-to-net adjustments reflecting actual rebate and return activity during the period. For the three months ended September 30, 2023, product revenue, net was primarily related to units of RELYVRIO and ALBRIOZA sold in the U.S. and Canada.

Cost of sales for the three months ended September 30, 2024 were primarily related to losses on firm purchase commitments. For the three months ended September 30, 2023, cost of sales consisted of costs to procure, manufacture and distribute units of RELYVRIO and ALBRIOZA in the U.S. and Canada.

Acquired In-process Research and Development Expenses

On July 9, 2024, we completed the Eiger Acquisition. During the three months ended September 30, 2024, we recorded a charge of approximately $36.2 million associated with the acquired in-process research and development assets of avexitide with no alternative future use.

Research and Development Expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2024 and 2023 (in thousands):

	Three Months Ended September 30,			
	2024	2023	$ Change	% Change
AMX0035 – ALS	$ 3,478	$ 12,955	$ (9,477)	(73)%
AMX0035 – PSP	4,995	1,571	3,424	218%
Payroll and personnel-related	7,464	11,788	(4,324)	(37)%
Other	5,300	3,723	1,577	42%
	$ 21,237	$ 30,037	$ (8,800)	(29)%

Research and development expenses were $21.2 million for the three months ended September 30, 2024, compared to $30.0 million for the three months ended September 30, 2023. The decrease was primarily due to a $9.5 million decrease in spending on AMX0035 for the treatment of ALS following the topline data from the PHOENIX trial, and a $4.3 million decrease in payroll and personnel-related primarily related to a decrease in the number of employees as a result of the Restructuring Plan. The decrease in

research and development expenses was offset by a $3.4 million increase in spending on AMX0035 for the treatment of PSP and a $1.6 million increase in other costs. The increase in spending on AMX0035 for the treatment of PSP was primarily related to costs to support the continuation of the ORION Phase 2b/3 global clinical trial, and the increase in other costs were primarily due to an increase in preclinical development activities.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $17.8 million for the three months ended September 30, 2024 compared to $48.7 million for the three months ended September 30, 2023. The decrease was primarily due to a decrease of $14.5 million in payroll and personnel-related costs, a decrease of $10.7 million in consulting and professional services and a decrease of $5.7 million in other expenses. The decrease in payroll and personnel-related costs was primarily related to a decrease in the number of employees as a result of the Restructuring Plan. The decrease in consulting and professional services is primarily due to a decrease in commercial sales and marketing activity as a result of the RELYVRIO®/ALBRIOZA™ Discontinuation. The decrease in other expenses is primarily due decrease in activity to wind down commercial operations.

Provision for Income Taxes

We recorded an income tax provision of zero and $1.5 million for the three months ended September 30, 2024 and 2023, respectively. The absence of an income tax provision for the three months ended September 30, 2024 was driven by the estimated effective tax rate for the year. The income tax benefit for the three months ended September 30, 2023 was primarily driven by the estimated annual effective tax rate for the year, as well as discrete income tax benefit of $0.1 million.

Comparison of the nine months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the periods presented (in thousands):

	Nine Months Ended September 30,			
	2024	2023	$ Change	% Change
Product revenue, net	$ 88,036	$ 272,337	$ (184,301)	(68)%
Operating expenses:				
Cost of sales	5,953	16,081	(10,128)	(63)%
Cost of sales - inventory impairment and loss on firm purchase commitments	118,680	—	118,680	*NM
Acquired in-process research and development	36,203	—	36,203	*NM
Research and development	81,192	83,273	(2,081)	(2)%
Selling, general and administrative	97,234	136,115	(38,881)	(29)%
Restructuring expenses	22,851	—	22,851	*NM
Total operating expenses	362,113	235,469	126,644	54%
(Loss) income from operations	(274,077)	36,868	(310,945)	(843)%
Other income, net:				
Interest income	11,493	11,784	(291)	(2)%
Other expense, net	(1,371)	(831)	(540)	65%
Total other income, net	10,122	10,953	(831)	(8)%
(Loss) income before income taxes	(263,955)	47,821	(311,776)	(652)%
Provision for income taxes	242	3,281	(3,039)	(93)%
Net (loss) income	$ (264,197)	$ 44,540	$ (308,737)	(693)%

Product Revenue, Net

Product revenue, net was $88.0 million for the nine months ended September 30, 2024, compared to $272.3 million for the nine months ended September 30, 2023. During these periods, product revenue, net was primarily related to units of RELYVRIO and ALBRIOZA sold in the U.S. and Canada, respectively. As a result of the RELYVRIO®/ALBRIOZA™ Discontinuation, we did not generate significant revenue from product sales in the second or third quarters of 2024.

Cost of Sales

Cost of sales were $124.6 million for the nine months ended September 30, 2024, compared to $16.1 million for the nine months ended September 30, 2023. During these periods, cost of sales consisted of costs to procure, manufacture and distribute our marketed products, RELYVRIO and ALBRIOZA. As a result of the RELYVRIO®/ALBRIOZA™ Discontinuation, the Company recorded approximately $118.7 million of charges associated with the write-down of inventory and losses on firm purchase commitments for the nine months ended September 30, 2024.

Acquired In-process Research and Development Expenses

On July 9, 2024, the Company completed the Eiger Acquisition. During the nine months ended September 30, 2024, the Company recorded a charge of approximately $36.2 million associated with the acquired in-process research and development assets of avexitide with no alternative future use.

Research and Development Expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2024 and 2023 (in thousands):

	Nine Months Ended September 30,			
	2024	2023	$ Change	% Change
AMX0035 – ALS	$ 25,577	$ 41,858	$ (16,281)	(39)%
AMX0035 – PSP	12,277	2,782	9,495	341%
Payroll and personnel-related	30,322	31,186	(864)	(3)%
Other	13,016	7,447	5,569	75%
	$ 81,192	$ 83,273	$ (2,081)	(2)%

Research and development expenses were $81.2 million for the nine months ended September 30, 2024, compared to $83.3 million for the nine months ended September 30, 2023. The slight decrease was primarily due to a $16.3 million decrease in spending on AMX0035 for the treatment of ALS following the topline data from the PHOENIX trial, and a $0.9 million decrease in payroll and personnel-related costs related to a decrease in the number of employees as a result of the Restructuring Plan. The decrease in research and development expenses was offset by a $9.5 million increase in spending on AMX0035 for the treatment of PSP and a $5.6 million increase in other costs due to an increase in preclinical development activities. The increase in spending on AMX0035 for the treatment of PSP was primarily related to costs to support the continuation of the ORION Phase 2b/3 global clinical trial.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $97.2 million for the nine months ended September 30, 2024 compared to $136.1 million for the nine months ended September 30, 2023. The decrease was primarily due to a decrease of $23.1 million in payroll and personnel-related costs and a decrease of $14.9 million in consulting and professional services, offset by an increase of $0.9 million in other expenses. The decrease in payroll and personnel-related costs was primarily related to a decrease in the number of employees as a result of the Restructuring Plan. The decrease in consulting and professional services is primarily due to a decrease in commercial sales and marketing activity as a result of the RELYVRIO®/ALBRIOZA™ Discontinuation. The increase in other expenses is primarily due to an increase in spending for operations as a public company and other expenses.

Restructuring Expenses

During the nine months ended September 30, 2024, restructuring expenses were approximately $22.9 million which includes employee severance and termination benefits of approximately $21.9 million, contract termination costs, impairment of long-lived assets and other costs of $1.0 million. We substantially completed the Restructuring Plan in the second quarter of 2024.

Provision for Income Taxes

We recorded an income tax provision of $0.2 million and an income tax benefit of $3.3 million for the nine months ended September 30, 2024 and 2023, respectively. The income tax provision for the nine months ended September 30, 2024 was driven by discrete income tax expense of $0.2 million. The income tax benefit for the nine months ended September 30, 2023 was primarily driven by the estimated effective tax rate for the year, as well as discrete income tax benefit of $0.5 million.

Liquidity and Capital Resources

Sources of Liquidity

In the second half of 2022, we began generating revenue from the sale of our approved drug product RELYVRIO, known as ALBRIOZA in Canada. In April 2024, we announced the RELYVRIO®/ALBRIOZA™ Discontinuation. We also announced the Restructuring Plan, which was designed to focus our resources on key clinical and preclinical programs and included a reduction in force which reduced our workforce by approximately 70% and decreased external financial commitments outside of our priority areas. We completed the Restructuring Plan in the second half of 2024. To date, we have financed our operations primarily through revenue from the sale of our approved products, the sale and issuance of common stock, convertible preferred stock and convertible notes. As of September 30, 2024, we had cash, cash equivalents and marketable securities of $234.4 million.

Based on our current operational plans and assumptions, we believe that our existing cash, cash equivalents, and marketable securities, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least twelve months after the date of the filing of this Quarterly Report. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect.

Capital Resources and Uses

We expect to incur significant expenses in connection with our ongoing activities, particularly as we advance the preclinical activities, manufacturing and clinical trials of AMX0035, avexitide and any other current or future product candidates or acquire or in-license additional product candidates or products. In addition, we expect to continue to incur costs associated with operating as a

public company, including significant legal, accounting, investor relations and other expenses. We expect to incur significant expenses as we:

- continue our research and development efforts, including our ongoing Phase 3 trial of AMX0035 in PSP and our ongoing Phase 2 trial of AMX0035 for the treatment of Wolfram syndrome;
- continue our research and development efforts of avexitide in PBH and conduct clinical trials of avexitide;
- continue to develop AMX0114, antisense oligonucleotide, for the treatment of people living with ALS;
- pursue INDs of AMX0035 for additional indications;
- conduct preclinical studies and clinical trials for AMX0035 for additional indications and for potential future product candidates;
- seek to identify and develop, acquire or in-license additional product candidates;
- experience any delays or encounter any issues with any of the above, including but not limited to failed studies, complex results, safety issues, or other regulatory challenges;
- develop the necessary processes, controls and manufacturing data to obtain additional marketing approval for AMX0035 or approval for any future product candidates and to support manufacturing on a commercial scale;
- seek additional regulatory approvals for AMX0035 or approvals for any future product candidates that successfully complete clinical trials, if any;
- incur expenses in preparation for commercialization for any approved product candidates related to product sales, marketing, manufacturing, and distribution;
- hire and retain additional personnel, such as preclinical, clinical, quality assurance, regulatory affairs, manufacturing, distribution, legal, compliance, finance, general and administrative, commercial and scientific personnel; and
- develop, maintain, expand and protect our intellectual property portfolio.

Because of the numerous risks and uncertainties associated with research, development and commercialization of product candidates and programs, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical and clinical development for AMX0035, avexitide and any future product candidates;
- the costs, timing and outcome of any future commercialization activities, including manufacturing, marketing, sales and distribution costs;
- the costs, timing and outcome of regulatory review of AMX0035, avexitide and any future product candidates;
- our ability to establish and maintain collaborations, marketing, distribution and license agreements on favorable terms, if at all;
- our ability to enroll clinical trials in a timely manner and to quickly resolve any delays or clinical holds that may be imposed on our development activities;
- timing delays with respect to preclinical and clinical development of AMX0035, avexitide and any future product candidates, including as result of any future outbreak of any highly infectious or contagious diseases;
- costs associated with identifying and developing, acquiring or in-licensing additional product candidates or products;
- the costs of any future expansion of our facilities to accommodate our potential growth in personnel, and the costs of such additional personnel;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;

•the extent to which we acquire technologies or other assets;

•the sales price and availability of adequate third-party coverage and reimbursement for any future product candidates, if and when approved;

•the costs of current and potential legal proceedings that may not be covered by our insurance; and

•the costs of operating as a public company.

Until such time, if ever, that we can generate product revenue sufficient to resume and sustain profitability, we may finance our cash needs through equity offerings, debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, current ownership interests will be diluted. If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Cash Flows

Comparison of the nine months ended September 30, 2024 and 2023

The following table summarizes our sources and uses of cash for the periods presented (in thousands):

	Nine Months Ended September 30,			
	2024	**2023**	**$ Change**	**% Change**
Net cash used in operating activities	$ (108,639)	$ (1,632)	$ (107,007)	*NM
Net cash provided by investing activities	10,674	71,128	(60,454)	(85)%
Net cash provided by financing activities	238	3,313	(3,075)	(93)%
Effect of exchange rate changes on cash, cash equivalents and restricted cash equivalents	87	(77)	164	(213)%
Net (decrease) increase in cash, cash equivalents and restricted cash equivalents	$ (97,640)	$ 72,732	$ (170,372)	(234)%

Operating Activities

During the nine months ended September 30, 2024, operating activities used $108.6 million of cash, primarily resulting from our net loss of $264.2 million, $8.1 million net accretion of discounts on investments and $19.1 million of net cash used by changes in our operating assets and liabilities, offset by $118.7 million of inventory impairment and loss on firm purchase commitments and $26.3 million of non-cash stock-based compensation expense. Our net loss non-cash adjustments also include $36.2 million of acquired IPR&D assets, which are classified as investing activities.

Net cash used by changes in our operating assets and liabilities primarily consisted of a $38.3 million decrease in accounts receivable, net, offset by a $9.3 million increase in inventory capitalized during the period, net of inventory written-off, a decrease of $33.4 million in accrued expenses and a $20.6 million decrease in accounts payable.

During the nine months ended September 30, 2023, operating activities used $1.6 million of cash, primarily resulting from our net income of $44.5 million and $27.6 million of non-cash stock-based compensation expense, offset by $7.4 million net accretion of discounts on investments and $67.2 million of net cash used by changes in our operating assets and liabilities.

Net cash used in our operating assets and liabilities primarily consisted of a $46.6 million increase in inventory capitalized during the period, a $14.0 million increase in accounts receivable, net, and a $7.6 million increase in prepaid expenses and other current assets.

Investing Activities

During the nine months ended September 30, 2024, net cash provided by investing activities was $10.7 million, resulting primarily from $279.0 million of investments that matured, offset by $232.0 million of purchases of marketable securities and a $36.2 million cash outflow to acquire IPR&D assets related to the avexitide asset acquisition.

During the nine months ended September 30, 2023, net cash provided by investing activities was $71.1 million, resulting primarily from $246.2 million of investments that matured, partially offset by $174.2 million of purchases of marketable securities during the period.

Financing Activities

During the nine months ended September 30, 2024, net cash provided by financing activities was $0.2 million. This amount consisted primarily of $0.2 million of proceeds from exercises of stock options and vesting of stock awards, net of withholding taxes paid on stock-based awards.

During the nine months ended September 30, 2023, net cash provided by financing activities was $3.3 million. This amount consisted primarily of $3.4 million of proceeds from exercises of stock options and vesting of stock awards, net of employee taxes paid on these awards.

Contractual Obligations and Commitments

We enter into agreements in the normal course of business with contract manufacturing organizations for raw material purchases and manufacturing services. As of September 30, 2024, we had approximately $27.2 million of remaining obligations under these agreements, which are expected to be paid through 2025.

Critical Accounting Policies, Recent Accounting Pronouncements and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the U.S. The preparation of our condensed consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

There have been no significant changes to our critical accounting policies from those described in "*Management's Discussion and Analysis of Financial Condition and Results of Operations*," disclosed in our 2023 Annual Report.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Market risk is the potential loss that may result from market changes associated with our business or with an existing or forecasted financial transactions. We are exposed to various market risks in the ordinary course of our business which are discussed below.

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of September 30, 2024 and December 31, 2023, we had cash, cash equivalents and marketable securities of $234.4 million and $371.4 million, respectively. Our cash equivalents are invested primarily in bank deposits and money market mutual funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Our investments are subject to interest rate risk and could fall in value if market interest rates increase. Due to the duration of our investment portfolio and the low risk profile of our investments, we do not believe an immediate 100 basis point change in interest rates would have a material effect on the fair market value of our portfolio. We have the ability to hold our investments until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

Foreign Currency and Currency Translation Risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with vendors that are located outside of the U.S. As a result, our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Inflation Risk

We do not believe that inflation had a material effect on our business, financial condition, or results of operations during the nine months ended September 30, 2024 and 2023. However, inflation has had, and may continue to have, an impact on the labor costs we incur to attract and retain qualified personnel.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We maintain “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms and (2) accumulated and communicated to our management, including our principal executive officers and principal financial officer, to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Our management, with the participation of our principal executive officers and our principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2024. Based on the evaluation of our disclosure controls and procedures as of September 30, 2024, our principal executive officers and principal financial officer concluded that, as of such date, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during the three months ended September 30, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting. While we experienced changes in personnel resulting from our Restructuring Plan, these changes did not necessitate changes in internal control process design or operation that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

On February 9, 2024, a putative class action lawsuit was filed in the U.S. District Court for the Southern District of New York against the Company and certain of our current and former officers (*Shih v. Amylyx Pharmaceuticals, Inc., et al.*, Case Number 1:24-CV-00988, or the Shih Complaint). Plaintiff filed an amended complaint on June 24, 2024. The Shih Complaint asserts a claim against all defendants for alleged violations of Section 10(b) of the Exchange Act and Rule 10b-5 promulgated thereunder and a claim under Section 20(a) against certain current and former officers as alleged controlling persons. The Shih Complaint alleges that defendants made materially false and misleading statements related to the commercial results and prospects for RELYVRIO. The Shih Complaint seeks unspecified damages, interest, costs and attorneys' fees, and other unspecified relief that the court deems appropriate. On August 12, 2024, the case was transferred from the U.S. District Court for the Southern District of New York to the U.S. District Court for the District of Massachusetts and assigned docket number 1:24-CV-12068. Following the transfer, on September 6, 2024, defendants moved to dismiss the Shih Complaint. Plaintiff filed his opposition to Defendants' motion to dismiss on October 21, 2024 and defendants' response is due on or before November 20, 2024.

On October 2, 2024, a derivative complaint was filed in the U.S. District Court for the District of Massachusetts, or the Court, against certain current and former director and officer defendants, naming the Company as nominal defendant (*Jones v. Cohen, et al*., 1:24-CV-12527, or the Derivative Complaint). The substantive allegations mirror those of the Shih Complaint but also include claims for alleged violations of Section 14(a) of the Exchange Act, breach of fiduciary duty, insider trading, and unjust enrichment. The Derivative Complaint seeks unspecified damages to be awarded to the Company along with interest, costs, and attorneys' fees, restitution, and unspecified corporate governance and internal procedural reforms and improvements. On October 31, 2024, the Court entered an order staying the action until the earlier of the dismissal of the Shih Complaint with prejudice, including the exhaustion of all appeals, or defendants file an answer to the Shih Complaint.

The Company intends to defend against the Shih Complaint and Derivative Complaint vigorously. At this time, an estimate of the impact, if any, of the claims made in the Shih Complaint and Derivative Complaint cannot be made.

Item 1A. Risk Factors.

Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Quarterly Report and in other documents that we file with the SEC, in evaluating our business and our prospects. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and are not the only risks that we face. New risk factors can emerge from time to time, and it is not possible to predict the impact that any factor or combination of factors may have on our business, prospects, financial condition and results of operations.

Summary of Risk Factors

Risks Related to Our Financial Position and Need for Capital

- We have ceased marketing and selling our only commercial product in the U.S. and Canada and will therefore not continue to generate revenue from this product for the treatment of ALS. We expect to generate significant losses for the foreseeable future.
- We will not return to profitability unless and until we successfully commercialize any of our current or future product candidates.
- We have a limited operating history and, following the withdrawal of RELYVRIO and ALBRIOZA, do not have any commercial products, which may make it difficult to evaluate the prospects for our future viability.
- We may require substantial additional funding in the future to meet our financial needs and to pursue our business objectives. If we are unable to obtain funding if and when needed, we could be forced to delay, reduce or eliminate our product discovery and development activities or commercialization efforts.

Risks Related to the Discovery and Development of Our Current and Future Product Candidates

- We currently depend heavily on the success of AMX0035, one of our most advanced product candidates and avexitide, our recently acquired product candidate. If we are unable to successfully complete late-stage trials, obtain regulatory

approvals for, and successfully commercialize, AMX0035 or avexitide, or experience significant delays in doing so, our business may be materially harmed.

•The delay or denial of regulatory approval for our current or any future product candidates in any jurisdiction could adversely impact our business and our results of operations, and could cause us delay or even cease operations.

•AMX0035 is a fixed-dose combination drug product and certain regulatory authorities may require a demonstration that each component makes a contribution to the claimed effects in addition to demonstrating that the combination is safe and effective for the intended population.

•We have historically concentrated our research and development efforts on the treatment of neurodegenerative and CNS disorders, a field that has seen very limited success in product development, and have only recently further expanded upon our existing development efforts in the endocrine and metabolic field.

•The regulatory approval processes of the FDA and other comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to maintain or obtain regulatory approval for any current or future product candidates, our business will be substantially harmed.

Risks Related to Our Dependence on Third Parties

•We may seek to establish collaborations and if we are not able to establish and maintain them on commercially reasonable terms, we may have to alter our development and future commercialization plans.

•We have entered and may in the future enter into collaborations with third parties for the development and commercialization of AMX0035, avexitide or any other current or future product candidates, and our prospects with respect to AMX0035, avexitide and our other current or future product candidates will depend in significant part on the success of those collaborations.

•We rely on third parties to assist in conducting our clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or successfully commercialize AMX0035, avexitide, or any other current or future product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.

Risks Related to Commercialization of AMX0035, Avexitide or Future Product Candidates

•The market for AMX0035 for Wolfram syndrome, PSP and other neurodegenerative diseases and of avexitide for post-bariatric hypoglycemia, or PBH, and congenital hyperinsulinism, or congenital HI, and for any other product candidates we are currently developing or may in the future develop or acquire may be smaller than we expect.

•If we are unable in the future to expand our sales, marketing, manufacturing and distribution capabilities or enter into agreements with third parties to market and sell AMX0035, avexitide or other current or future product candidates for which we obtain marketing approval, we will be unable to generate any additional product revenue.

•Even if any future product candidate of ours receives regulatory approval, it may fail to maintain the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for continued commercial success or to remain profitable.

Risks Related to Our Intellectual Property

•Our commercial success depends on our ability to protect our intellectual property and proprietary technology.

Risks Related to Our Business Operations and Employee Matters

•Our Restructuring Plan and associated organizational changes may not adequately reduce our operating costs or improve operating margins, may lead to additional workforce attrition, and may cause operational disruptions.

•We are currently operating in a period of economic uncertainty and capital markets disruption, which has been significantly impacted by geopolitical instability, ongoing military conflicts, including the ongoing war between Russia and Ukraine, the Israel-Hamas war and escalating conflict in the Middle East, U.S. presidential elections, events related thereto, such as changes to candidates or political unrest or otherwise, and high inflation and interest rates, any of which could have a material adverse effect on our business, financial condition and results of operations.

•We depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.

•We only have a limited number of employees to manage and operate our business.

•A pandemic, epidemic, or outbreak of an infectious disease may materially and adversely affect our business, including our preclinical studies, clinical trials, third parties on whom we rely, our supply chain, our ability to raise capital, our ability to conduct regular business and our financial results.

•We are continuously evaluating and pursuing strategic transactions, and may pursue strategic transactions in the future that are aligned with our mission in to improve our underlying business performance. For example, we have recently completed the acquisition of avexitide, and may in the future seek to acquire additional assets.

Risks Related to Our Common Stock

•Unstable market, economic, political and geographical conditions may have serious adverse consequences on our business, financial condition and stock price.

Risks Related to Our Financial Position and Need for Capital

We have ceased marketing and selling our only commercial product in the U.S. and Canada and will therefore not continue to generate revenue from this product for the treatment of ALS. We expect to generate significant losses for the foreseeable future.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have invested substantial resources into our product development efforts of AMX0035, and toward the commercialization of AMX0035 as RELYVRIO for ALS, which was approved by the FDA, and ALBRIOZA, which received marketing authorization with conditions from Health Canada. We voluntarily discontinued the marketing authorizations for RELYVRIO/ALBRIOZA(AMX0035) for ALS and remove the product from the market in the U.S. and Canada based on topline results from the Phase 3 PHOENIX trial, which failed to meet its prespecified primary and secondary endpoints. Following the withdrawal of RELYVRIO/ALBRIOZA from the market in the US and Canada, respectively, we do not have any products approved for commercial sale and we will continue to incur significant research and development and other expenses related to clinical development and potential approvals for our current and future product candidates, including for AMX0035 in additional indications other than ALS, for avexitide, and for ongoing operations. Since our inception, we have devoted the majority of our financial resources and efforts to research and development, including preclinical studies and our clinical trials, preparation for commercialization and commercialization activities. Our financial condition and operating results, including our revenues, expenses and net income (loss), have in the past and are likely in the future to fluctuate significantly from quarter to quarter and year to year. For example, we generated revenues of $380.8 million in 2023 as a result of sales of RELYVRIO/ALBRIOZA, but following its withdrawal, we will no longer generate revenues from this product. Accordingly, you should not rely upon the results of any prior quarterly or annual periods as indications of future operating performance. Additionally, net losses and negative cash flows have had, and may in the future have, an adverse effect on our stockholders' equity and working capital. As of September 30, 2024, we had an accumulated deficit of $569.1 million. We expect to incur significant losses for the foreseeable future.

We anticipate that significant expenses will be incurred if and as we:

- conduct clinical trials of AMX0035, avexitide, and any other current or future product candidates;
- seek to identify and advance additional product candidates;
- initiate and continue research, preclinical and clinical development efforts for any current or future product candidates;
- maintain, expand and protect our intellectual property portfolio;
- seek regulatory approvals for AMX0035 or avexitide in indications that successfully complete clinical development; and
- acquire or in-license other product candidates, products or technologies.

We will not return to profitability unless and until we successfully commercialize any of our current or future product candidates.

Our ability to become and remain profitable depends on our ability to generate revenue. While we have a limited history of generating revenue from the commercialization of RELYVRIO/ALBRIOZA, we do not expect to generate significant revenue, if any, unless and until we, either alone or with a collaborator, are able to obtain regulatory approval for, and successfully commercialize, AMX0035 for indications other than ALS, avexitide or any other current or future product candidates or products we may develop or in-license. Successful commercialization will require achievement of many key milestones, which vary by jurisdiction and may include demonstrating safety and efficacy in clinical trials, obtaining regulatory, including marketing, approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our future collaborators, may obtain regulatory approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately and precisely predict the timing and amount of revenues, the extent of any further losses or if or when we might achieve profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our failure to regain profitability may continue to depress the market price of our common stock and could impair our ability to raise capital or obtain other financing, expand our business, diversify our product offerings or continue our operations. If we continue to suffer losses as we have in the past, prior to the commercialization of RELYVRIO and ALBRIOZA, investors may not receive any return on their investment and may lose their entire investment.

We have a limited operating history and, following the withdrawal of RELYVRIO and ALBRIOZA, do not have any commercial products, which may make it difficult to evaluate the prospects for our future viability.

Our operations to date have been primarily limited to organizing, staffing and financing our company, raising capital, conducting research and development activities, including preclinical studies and clinical trials, prior to discontinuation, preparing for and commercializing AMX0035 for the treatment of ALS and, more recently, restructuring, reprioritizing our assets and acquiring and incorporating avexitide into our pipeline. Accordingly, you should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in clinical development, especially clinical-stage biopharmaceutical companies such as ours. Any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or if we continued to have a marketed product.

We may require substantial additional funding in the future to meet our financial needs and to pursue our business objectives. If we are unable to obtain funding if and when needed, we could be forced to delay, reduce or eliminate our product discovery and development activities or commercialization efforts.

Our operations have consumed substantial amounts of cash since inception. We expect to spend substantial amounts to continue the clinical development of AMX0035 in indications other than ALS, for the clinical development of avexitide and for the preclinical and clinical development of additional product candidates, or in the in-license, acquisition or development of other product candidates or products. If we are unable to obtain additional marketing approvals for AMX0035, for avexitide or for any other current or future product candidates that we develop, in-license or acquire, we may require significant additional amounts of cash in order to continue to develop AMX0035, avexitide and any other current or future product candidates and fund our operations. In addition, other unanticipated costs may arise in the course of our development efforts. Because the design and outcome of our ongoing and anticipated clinical trials is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of any product candidate we develop.

Our future capital requirements depend on many factors, including:

- the scope, progress, results and costs of researching and developing AMX0035 for Wolfram syndrome, PSP and potential additional indications, avexitide as well as any other product candidates we are currently developing or may in the future develop;
- the timing of, and the costs involved in, obtaining marketing approvals for AMX0035 for the treatment of Wolfram syndrome, PSP and potential additional indications and avexitide, and obtaining approvals for other product candidates we are developing or may in the future develop and pursue;
- the number of other product candidates that we may pursue and their development requirements;
- the costs of commercialization activities for AMX0035 and avexitide for any approved indications, or any other product candidate that receives regulatory approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing sufficient product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of regulatory approval on a jurisdiction-by-jurisdiction basis, revenue, if any, received from commercial sales of AMX0035 and avexitide for any approved indications or any other current or future product candidates;
- the extent to which we in-license or acquire rights to other products, product candidates or technologies;
- our obligation, if any, to pay royalties in connection with the development and commercialization of avexitide;
- our headcount fluctuation;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights, including enforcing and defending intellectual property related claims; and
- the ongoing costs of operating as a public company.

We cannot be certain that additional funding will be available on acceptable terms, or at all. As a result of the challenges caused by economic uncertainty in various global markets due to geopolitical instability and conflict, including the ongoing wars in Ukraine and Israel, the escalating conflict in the Middle East, the presidential elections in the United States, the global credit and financial markets have experienced in recent periods significant volatility and disruptions, including diminished liquidity and credit availability, declines in consumer confidence, high rates of inflation and interest rates and uncertainty about economic stability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly or more dilutive.

We have no committed source of additional capital and if we are unable to raise additional capital or secure other financing, if needed, in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development of AMX0035, avexitide or any other current or future product candidates or other research and development initiatives. We may need to seek collaborators for AMX0035, avexitide and any other current or future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to AMX0035, avexitide and any other current or future product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves. Any of the above events could significantly harm our business, prospects, financial condition, and results of operations and cause the price of our common stock to decline.

We believe that our existing cash, cash equivalents, and marketable securities, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least twelve months after the date of this Quarterly Report. However, our estimate may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We expect our expenses to continue to increase in connection with our planned operations. Unless and until we can generate a substantial amount of revenue on a sustained basis, if at all, we may be required to finance our future cash needs through public or private equity offerings, royalty-based or debt financings, collaborations, licensing arrangements or other sources, or any combination of the foregoing. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans.

To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, your ownership interest may be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. Securing financing could also require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of AMX0035, avexitide or any future product candidates.

If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us.

Changes in tax law could adversely affect our business and financial condition.

The rules dealing with U.S. federal, state, local and international income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults or non-performance by financial institutions or transactional counterparties, could adversely affect our current and projected business operations and our financial condition and results of operations.

Events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. Although we do not currently have investments with any financial institution that has experienced such events, if any financial institution with which we have a relationship were to be placed into receivership, we may be unable to access such funds. In addition, if any parties with whom we conduct business are unable to access funds pursuant to instruments or lending arrangements with such a financial institution, such parties' ability to pay their obligations to us or to enter into new commercial arrangements requiring additional payments to us could be adversely affected.

Inflation and rapid increases in interest rates have led to a decline in the trading value of previously issued government securities with interest rates below current market interest rates. Although the U.S. Department of Treasury, FDIC and Federal Reserve Board have announced a program to provide up to $25 billion of loans to financial institutions secured by certain government securities held by financial institutions to mitigate the risk of potential losses on the sale of such instruments, widespread demands for customer withdrawals or other liquidity needs of financial institutions for immediate liquidity may exceed the capacity of such program. Additionally, there is no guarantee that the U.S. Department of Treasury, FDIC and Federal Reserve Board will provide access to uninsured funds in the event of the closure of other banks or financial institutions in the future, or that they would do so in a timely fashion.

Although we assess our banking relationships as we believe necessary or appropriate, our access to funding sources in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect us, the financial institutions with which we have financial arrangements directly, or the financial services industry or economy in general. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry.

In addition, investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our financial and/or contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity and our current and/or projected business operations and financial condition and results of operations.

Risks Related to the Discovery and Development of Our Current and Future Product Candidates

We currently depend heavily on the success of AMX0035, one of our most advanced product candidates, and avexitide, our recently acquired product candidate. If we are unable to successfully complete clinical trials for, obtain regulatory approvals for, and successfully commercialize, AMX0035 or avexitide, or experience significant delays in doing so, our business may be materially harmed.

Our future success depends significantly on our ability to successfully develop, and obtain regulatory approvals for and commercialize, AMX0035 in indications other than ALS, including Wolfram syndrome and PSP, and avexitide. To date, we have obtained limited clinical trial data supporting AMX0035 in indications other than ALS, having only completed a clinical trial in 95 patients with AD. We are conducting a Phase 2 clinical trial of AMX0035 in Wolfram syndrome, and a global Phase 3 clinical trial of AMX0035 in PSP, and intend to conduct additional clinical trials for other indications and product candidates in the future. Our business success depends heavily on our ability to successfully complete clinical trials for our product candidates. In connection with the Eiger Acquisition, we acquired substantially all of the rights, title and interests in, to avexitide, an investigational, first-in-class GLP-1 receptor antagonist that has been evaluated in five Phase 2 clinical studies for PBH and Congenital HI. We expect to begin a Phase 3 program for avexitide for PBH in the first quarter of 2025. We are also conducting IND enabling studies of AMX0114 in ALS and plan to initiate the Phase 1 multiple ascending dose, placebo-controlled trial in Canada by the end of 2024 or in early 2025.

We will need to have sufficient funds for, and successfully complete, our clinical development of AMX0035 for the treatment of PSP, Wolfram syndrome and other indications, avexitide and AMX0114 in ALS.

The future regulatory and commercial success of AMX0035, avexitide or any other current or future product candidates are subject to a number of risks, including the following:

- successful completion of preclinical studies and clinical trials;
- successful patient enrollment in clinical trials;
- successful data from our preclinical studies and clinical trials that supports an acceptable risk-benefit profile of AMX0035, avexitide or any other current or future product candidates in the intended populations;
- satisfaction of applicable regulatory requirements, including to satisfy applicable rules governing fixed dose combination products, as applicable;
- the interpretation of our preclinical and clinical data by regulatory authorities to support marketing approvals;

•potential unforeseen safety issues or adverse side effects;

•receipt and maintenance of marketing approvals from applicable regulatory authorities, including with any expected NCE and new clinical investigation data exclusivity and orphan drug market exclusivity, as applicable;

•receipt and maintenance of designations from applicable regulatory authorities, including breakthrough designation for avexitide and orphan designation for AMX0035 and avexitide;

•obtaining and maintaining patent and trade secret protection and regulatory exclusivity for AMX0035, avexitide or any other current or future product candidates;

•making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of AMX0035, avexitide or any other current or future product candidates;

•entry into collaborations to further the development of AMX0035, avexitide or any other current or future product candidates;

•establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;

•acceptance of AMX0035, avexitide or any other products, if and when approved, by patients, the medical community and third-party payors;

•appropriately identifying patients with the diseases targeted by AMX0035, avexitide or any other current or future product candidates;

•obtaining and maintaining third-party coverage and adequate reimbursement;

•maintaining a continued acceptable safety profile of products following any approval;

•effectively competing with other drugs or therapies;

•ensuring that we promote and distribute our products consistent with all applicable healthcare laws; and

•enforcing and defending intellectual property rights and claims.

Many of these risks are beyond our control, including the risks related to clinical development, the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, obtain or maintain regulatory approvals for, or successfully commercialize AMX0035, avexitide or any of our other current or future product candidates, or if we experience delays as a result of any of these risks or otherwise, our business could be materially harmed.

In addition, of the large number of drugs in development in the pharmaceutical industry, only a small percentage result in the submission of marketing applications to regulatory authorities, and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval for our current or any of our future product candidates, any such approval may be subject to limitations on the indications or uses or the patient populations for which we may market the product. Additionally, we may not realize the full commercial potential of AMX0035, avexitide or any other current or future product candidates that receive marketing approval if we are unable to appropriately identify patients with the diseases targeted by such product candidates. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development activities, we cannot assure you that we will successfully develop or commercialize our current or any future product candidates for any indication in any jurisdiction. If we or any of our future collaborators are unable to develop, maintain, or obtain regulatory approvals for, or, if approved, successfully commercialize our current or any future product candidates for our initial or potential additional indications, we may not be able to generate sufficient revenue to continue our business. In addition, our failure to demonstrate positive results in our clinical trials in any indication for which we are developing our current product candidates, or to satisfy other regulatory requirements, could adversely affect our development efforts for AMX0035 in other indications, avexitide or for AMX0114.

The delay or denial of regulatory approval for our current or any future product candidates in any jurisdiction could adversely impact our business and our results of operations, and could cause us to delay or even cease operations.

The research, testing, manufacturing, labeling, approval, sale, marketing, distribution and post-market obligations of drug products are subject to extensive regulation by the FDA and other regulatory agencies in the U.S. and other countries, and such regulations differ from country to country.

The FDA or any other foreign regulatory agency can delay, limit, deny or withdraw approval to market AMX0035, avexitide or any future product candidates for many reasons, including:

- our inability to demonstrate to the satisfaction of, the FDA or any other applicable foreign regulatory agency that AMX0035, avexitide or any future product candidate is safe and effective for the requested indication;
- our inability to gain agreement from applicable foreign regulatory authorities that AMX0035, avexitide or any future product candidate is appropriate for approval under applicable regulatory pathways;
- the FDA's or any other applicable foreign regulatory agency's disagreement with the interpretation of data from preclinical and clinical studies and trials;
- our inability to demonstrate that the clinical and other benefits of AMX0035, avexitide or any future product candidate outweigh any safety or other perceived risks;
- the FDA's or any other applicable foreign regulatory agency's requirement for additional preclinical or clinical studies or trials, including studies to satisfy applicable rules governing fixed dose combination products or post-market requirements, as applicable;
- the FDA's or any other applicable foreign regulatory agency's having differing requirements for the trial protocols used in our clinical trials;
- the FDA's or any other applicable foreign regulatory agency's non-approval of the formulation, labeling and/or the specifications of AMX0035, avexitide or any future product candidates;
- the FDA's or any other applicable foreign regulatory agency's failure to accept the manufacturing processes or third-party manufacturers with which we contract; or
- the potential for approval policies or regulations the FDA or any other applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of drugs in development, only a small percentage successfully complete the FDA other regulatory approval processes and are commercialized.

The FDA or the applicable foreign regulatory agency may also approve AMX0035, avexitide or any future product candidates for a more limited indication and/or a narrower patient population than we originally request, and the FDA or any other applicable foreign regulatory agency may not approve the labeling that we believe is necessary or desirable for the successful commercialization of AMX0035, avexitide or any future product candidates. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of AMX0035, avexitide or any future product candidates and would materially adversely impact our business and prospects.

AMX0035 is a fixed-dose combination drug product and certain regulatory authorities may require a demonstration that each component makes a contribution to the claimed effects in addition to demonstrating that the combination is safe and effective for the intended population.

Under the FDA's combination rule, the FDA may not file or approve an NDA for a fixed-dose combination product unless each component of a proposed drug product is shown to make a contribution to the claimed effects and the dosage of each component (amount, frequency, duration) is safe and effective for the intended population. For additional information on FDA's combination rule, see the section entitled "*Business—Government Regulation—Combination Rule for Fixed-Dose Combination Products*" in our 2023 Annual Report.

Similar requirements may be imposed on us by the EMA in the EU and comparable regulatory authorities in other jurisdictions where we intend to seek regulatory approval. See the section entitled "*Business—Government Regulation—Fixed-Dose Combination Guideline*" in our 2023 Annual Report. For any fixed-dose combination products we may develop, we may be required to produce clinical data supporting the contribution of each component when present at the levels included in the fixed-dose combination in order to obtain marketing authorization in the U.S. or EU.

While the FDA approved AMX0035 (known as RELYVRIO) as a fixed-dose combination product for the treatment of ALS in adults, we may be required by the FDA and comparable foreign regulatory authorities to satisfy the fixed-dose combination rule for AMX0035 or any other fixed-dose combination products we may develop for the treatment of any other indications we may pursue in advance of approval.

If the FDA or other comparable foreign regulatory authorities require us to conduct one or more clinical trials to support such a demonstration, such as a factorial study, the design, duration, and scope of such clinical trials will be decided upon after further discussions with those agencies and other comparable foreign regulatory authorities. As a result, we are unable to predict with certainty the estimated timing or scope of any future clinical trials of AMX0035 we may be required to conduct to satisfy these requirements governing fixed dose combination products in various jurisdictions. Ongoing third-party data in neurology, specifically within ALS, on our products or other products may influence regulatory decision making, including for fixed-dose combinations.

We have historically concentrated our research and development efforts on the treatment of neurodegenerative and CNS disorders, a field that has seen very limited success in product development, and have only recently further expanded upon our existing development efforts in the endocrine and metabolic field.

We have focused our research and development efforts on addressing neurodegenerative and CNS disorders and have only recently further expanded upon our existing development efforts in the endocrine and metabolic field with the acquisition of avexitide. Thus, this shift in focus may result in additional costs arising from operating expenses and hiring personnel, challenges with building our expertise in the endocrine and metabolic field, or diversion of management's attention away from AMX0035. Historically, efforts by pharmaceutical companies in the field of neurodegenerative and CNS disorders have experienced limited successes in product development. The development of neurodegenerative and CNS therapies presents unique challenges, including an imperfect understanding of the biology, the presence of the blood brain barrier that can restrict the flow of drugs to the brain, a frequent lack of translatability of preclinical study results in subsequent clinical trials and dose selection, and the product candidate having an effect that may be too small to be detected using the outcome measures selected in clinical trials or if the outcomes measured do not reach statistical significance. There are few approved therapeutic options available for patients with ALS and other neurodegenerative disorders. Our future success is highly dependent on the successful development and commercialization of AMX0035, avexitide and any other current or future product candidates for treating neurodegenerative and CNS disorders or for treating endocrine and metabolic disorders. Developing and commercializing AMX0035, avexitide and any other current or future product candidates for treatment of neurodegenerative and CNS disorders or for treating PBH and Congenital HI subjects us to a number of challenges, including ensuring that we have selected the optimal doses, executing an appropriate clinical trial to test for efficacy and obtaining and maintaining regulatory approval from the FDA and other comparable foreign regulatory authorities.

The regulatory approval processes of the FDA and other comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our current or any future product candidates, our business will be substantially harmed.

We, and any future collaborators, are not permitted to commercialize, market, promote or sell any product candidate in the U.S. or elsewhere without obtaining regulatory approval from the FDA and other comparable foreign regulatory authorities. Regulatory authorities in other jurisdictions may have similar requirements. The time required to obtain approval by the FDA and other comparable foreign regulatory authorities is unpredictable, and typically takes many years following the commencement of clinical trials and depends upon numerous factors, including substantial discretion of such regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. For example, the U.S. Supreme Court's July 2024 decision to overturn prior established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which FDA's regulations, policies, and decisions may become subject to increasing legal challenges, delays, and/or changes. In addition, the FDA in any approval needs to determine that there is substantial evidence of effectiveness. This finding can be substantiated based on two adequate and well-controlled studies, or in certain circumstances on a single, large, multicenter, adequate and well-controlled study that is very persuasive or from a single adequate and well-controlled study together with confirmatory evidence. FDA regulations and guidance also allow for greater flexibility and tolerance for uncertainty in the context of rare and fatal diseases.

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of AMX0035 for our indications other than ALS, avexitide or any current or future product candidates is susceptible to the risk of failure inherent at any stage of development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements, and determination by the FDA or any other comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. For example, our Phase 3 clinical trial of AMX0035 for the treatment of ALS failed to meet its primary and secondary endpoints. Additionally, our expenses could increase if we are required by the FDA or any other comparable foreign regulatory authority to perform clinical trials in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of AMX0035 in additional indications, avexitide and any current or future product candidates. It is possible that even if AMX0035, avexitide or any other current or future product candidate has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more

of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of AMX0035, avexitide or any other current or future product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials we may fail to detect toxicity of or intolerability caused by AMX0035, avexitide or any other current or future product candidate, or mistakenly believe that AMX0035, avexitide or any other current or future product candidates are toxic or not well-tolerated when that is not in fact the case.

AMX0035, avexitide and any current or future product candidates could fail to obtain regulatory approvals, and any of our future product candidates could fail to obtain regulatory approvals, for many reasons, including the following:

- the FDA or other comparable foreign regulatory authorities may disagree as to the design or implementation of our clinical trials and may require additional data to support regulatory approval;
- we may be unable to demonstrate to the satisfaction of the FDA or other comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication(s) and, if necessary, that a product candidate and any active components thereof are safe and effective for the proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or other comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA and comparable authorities in other countries may disagree with our interpretation of data from clinical trials or preclinical studies and our request may require additional trials or studies to support marketing approval;
- the data collected from clinical trials of AMX0035, avexitide or any other current or future product candidates may not be sufficient to support the submission of an NDA or other submission to the FDA or other comparable foreign regulatory authority to obtain regulatory approval in the U.S. or elsewhere;
- the FDA or other comparable foreign regulatory authorities may find deficiencies with clinical trial sites or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or other comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of clinical trial results may result in our failing to obtain regulatory approval to market AMX0035, avexitide or any future product candidate we develop, which would significantly harm our business, results of operations and prospects. There is no assurance that the endpoints and trial designs used for the approval of currently approved drugs for the treatment of neurodegenerative diseases will be acceptable for future approvals, including for AMX0035, avexitide and any current or future product candidates. Similarly, there is no assurance that the endpoints and trial designs for avexitide will be acceptable for its future approval. The FDA and other comparable foreign authorities have substantial discretion in the approval process and determining when or whether regulatory approval will be obtained for any product candidate that we develop. Even if we believe the data collected from past or future clinical trials of AMX0035, avexitide or any other current or future product candidates are promising, such data may not be sufficient to support approval by the FDA or any other regulatory authority.

The FDA reviews an NDA to determine whether the product is safe and effective for its intended use(s), with the latter determination being made on the basis of substantial evidence. This finding can be substantiated based on two adequate and well-controlled studies, or in certain circumstances on a single, large, multicenter, adequate and well-controlled study that is very persuasive or from a single adequate and well-controlled study together with confirmatory evidence. The FDA may not agree that this standard is met. Accordingly, there can be no assurance that for AMX0035, avexitide or any other current or future product candidates the FDA and other regulatory agencies will not require additional clinical trials beyond what we may plan to conduct.

In addition, disruptions caused by any future public health crisis may increase the likelihood that we encounter difficulties or delays in initiating, screening, enrolling, conducting, or completing our ongoing and planned preclinical studies and clinical trials. Clinical site initiation and patient screening and enrollment may be delayed due to prioritization of hospital resources in the event of a future public health crisis. Investigators and patients may not be able to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services. Similarly, our ability to recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to such future highly infectious or contagious diseases, could be limited, which in turn could adversely impact our clinical trial operations. Additionally, we may experience interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with any future outbreak of any highly infectious or contagious

diseases. As a result of a future public health crisis, we may face delays in meeting our anticipated timelines for our ongoing and planned clinical trials.

In addition, regulatory authorities may subject our clinical or manufacturing operations to inspections, including routine surveillance, bioresearch monitoring and pre-approval inspections. In addition, even if we were to obtain approval, regulatory authorities may approve AMX0035, avexitide or any other current or future product candidates for fewer or more limited indications than we request, may not approve the price we intend to charge for our products, may grant approval contingent on the performance of costly post-marketing preclinical studies and clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could materially harm the commercial prospects for AMX0035, avexitide or any other current or future product candidates.

We may incur unexpected costs or experience delays in completing, or ultimately be unable to complete, the development of AMX0035, avexitide or any other current or future product candidates.

To obtain regulatory approval to commercialize AMX0035, avexitide and any other current or future product candidates, we must demonstrate through extensive preclinical studies and clinical trials that such product candidates are safe and effective in humans. Preclinical and clinical testing are expensive and can take many years to complete, and their outcome is inherently uncertain. Failure can occur at any time during the clinical trial process and our future clinical trial results may not be successful, which could impact our ability to obtain regulatory approvals for AMX0035, avexitide or any other current or future product candidates.

We may experience delays in completing our clinical trials or preclinical studies and initiating or completing additional clinical trials. We may also experience numerous unforeseen events during our clinical trials that could delay or prevent our ability to receive marketing approval or commercialize AMX0035, avexitide or any other current or future product candidates we develop, including:

- regulators, or institutional review boards, or IRBs, or other reviewing bodies may not authorize us or our investigators to commence a clinical trial, or to conduct or continue a clinical trial at a prospective or specific trial site;
- we may not reach agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the number of subjects or patients required for clinical trials of AMX0035 in an indication, avexitide or any future product candidate may be larger than we anticipate, enrollment in these clinical trials may be insufficient or slower than we anticipate, and the number of clinical trials being conducted at any given time may be high and result in fewer available patients for any given clinical trial, or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors, including those manufacturing AMX0035, avexitide or any other current or future product candidates or conducting clinical trials on our behalf, may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may have to amend clinical trial protocol submitted to regulatory authorities or conduct additional studies to reflect changes in regulatory requirements or guidance, which we may be required to resubmit to an IRB and regulatory authorities for re-examination;
- unforeseen safety events may occur during the course of a clinical trial and these events may result in the temporary suspension or termination of a clinical trial, or require urgent safety measures or restrictions to protect human subjects during the conduct of a clinical trial;
- regulators, IRBs or other reviewing bodies may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we enter into agreement for clinical and commercial supplies, or the supply or quality of AMX0035, avexitide or any other current or future product candidate or other materials necessary to conduct clinical trials of AMX0035, avexitide or any other current or future product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and
- the potential for approval policies or regulations of the FDA or any other applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Regulators, IRBs of the institutions in which clinical trials are being conducted or data monitoring committees may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

For example, we submitted an Investigational New Drug application to the FDA for AMX0114. The FDA restricted dosing to an amount that is lower than the Company's proposed starting dose of 12.5 mg and has requested additional information, which resulted in a clinical hold. Toxicology studies showed a greater than 10X safety margin at the starting dose of 12.5 mg based on the no observed adverse effect level (NOAEL) determined by independent toxicology firms. We are working to address FDA comments. We believe the trial can be completed outside of the U.S if needed.

Negative or inconclusive impressions of the results from our earlier clinical trials of AMX0035 for the treatment of ALS or AD, the results from earlier clinical trials of avexitide performed by Eiger or any other clinical trial or preclinical studies in animals that we or Eiger, with respect to avexitide, have conducted, could mandate repeated or additional preclinical studies or clinical trials and could delay marketing approvals or result in changes to or delays in preclinical studies or clinical trials of AMX0035 in other indications. We do not know whether any clinical trials that we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market AMX0035 for our intended indications, avexitide or any future product candidate.

Our failure to successfully initiate and complete clinical trials of AMX0035 for Wolfram syndrome, PSP, or potential additional indications or avexitide and to demonstrate the efficacy and safety of AMX0035 and avexitide, including each component thereof, necessary to obtain regulatory approval to market AMX0035 and avexitide, would significantly harm our business and ability to continue developing and marketing AMX0035 and avexitide for any indications. Our product candidate development costs will also increase if we experience delays in testing or obtaining and maintaining regulatory approvals and we may be required to obtain additional funds to complete clinical trials. We cannot assure you that our clinical trials will begin as planned or be completed on schedule, if at all, or that we will not need to restructure our trials after they have begun. Significant clinical trial delays or the need for additional data from our clinical trials also could shorten any periods during which we may have the exclusive right to commercialize AMX0035, avexitide or any other current or future product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize such product candidates, which may harm our business and results of operations. In addition, many of the factors that cause, or lead to, delays of clinical trials may ultimately lead to the denial of regulatory approval of AMX0035, avexitide or any future product candidate.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us or any future collaboration partners from obtaining or maintaining approvals for the commercialization of our current or any future product candidate we develop.

Any product candidate we may develop and the activities associated with its development and commercialization, including its design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the U.S. and in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing that product candidate in a given jurisdiction. Although we have invested substantial time and resources to date in pursuit of regulatory approval and toward potential commercialization, we have only received regulatory approval for AMX0035 (RELYVRIO) in the U.S. and marketing authorization with conditions for AMX0035 (ALBRIOZA) in Canada, which products we have since ceased marketing and selling from the market, and have not received any other regulatory approvals to market any product candidates from regulatory authorities in any jurisdiction, and it is possible that none of the product candidates we may seek to develop in the future will ever obtain regulatory approval. We have no experience in filing and supporting the applications necessary to gain marketing approvals and rely on third-party CROs or regulatory consultants to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate’s safety, purity, efficacy and potency. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we develop may not be effective, may be only moderately effective, or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, in the U.S. and other foreign jurisdictions, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity, and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide during the review process that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit, or prevent marketing approval of, or limit the approved labeling for, a product candidate. Additionally, the FDA has discretion to refer an application for a novel drug or a drug that presents difficult questions of safety or efficacy to an advisory committee. For example, our approval of RELYVRIO in the U.S. underwent two Advisory Committee reviews, which delayed ultimate approval.

If we experience delays in obtaining approval or if we fail to maintain or obtain approval of AMX0035, avexitide or of any product candidates we may develop, the commercial prospects for those product candidates, including for AMX0035 or avexitide, may be harmed, and our ability to generate revenues will be materially impaired.

The results of early-stage clinical trials and preclinical studies may not be predictive of future results. Initial or preliminary data in our clinical trials may not be indicative of results obtained when these trials are completed or in later stage trials.

The results of preclinical studies may not be predictive of the results of clinical trials, and the results of any early-stage clinical trials we commence may not be predictive of the results of the later-stage clinical trials. In addition, initial data in clinical trials may not be indicative of results obtained when such trials are completed. There can be no assurance that any of our clinical trials will ultimately be successful or support further clinical development of AMX0035, avexitide or any other current or future product candidates. For example, the clinical results seen in the CENTAUR trial were different than the results seen in our global Phase 3 PHOENIX clinical trial. There is a high failure rate for drugs and biologics proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving promising results in earlier studies, and any such setbacks in our clinical development could have a material adverse effect on our business and operating results.

Additionally, we have in the past utilized and may in the future utilize an “open-label” clinical trial design. An “open-label” clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a “patient bias” where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an “investigator bias” where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with AMX0035, avexitide or any future product candidates when studied in a controlled environment with a placebo or active control.

Interim topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim topline or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our reputation and business prospects.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

Patient enrollment is a significant factor in the timing of clinical trials, and the timing of our clinical trials depends, in part, on the speed at which we can recruit patients to participate in our trials, as well as completion of required follow-up periods. We may not be able to initiate or continue clinical trials for AMX0035, avexitide or any other current or future product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials to such trial’s conclusion as required by the FDA or other comparable foreign regulatory authorities. Additionally, certain clinical trials for AMX0035, avexitide and any other current or future product candidates may be focused on indications with relatively small patient populations, which may further limit enrollment of eligible patients or may result in slower enrollment than we anticipate. The eligibility criteria of our clinical trials, once established, may further limit the pool of available trial participants. For example the number of patients suffering from Wolfram syndrome and PBH, is small and, in some cases, has not been established with precision. If the actual number of patients with these diseases is smaller than we anticipate, we may encounter difficulties in enrolling patients in our clinical trials, thereby delaying or preventing development and approval of AMX0035, avexitide or any other current or future product candidates. Even once enrolled, we may be unable to retain a sufficient number of patients to complete any of our trials. Neurodegenerative diseases have particular challenges, including significant mobility issues, morbidities and other complications that have historically made retention in clinical trials more challenging. In the past, we have had discontinuations in our clinical trials, including in our CENTAUR trial and our PHOENIX trial, and their open label extensions. Discontinuations may occur in current or future trials and could result in delays of

completion of our clinical trials and affect our ability to enroll additional patients in our clinical trials and impact the integrity of data from our clinical trials.

Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the severity of the disease under investigation, the nature of the trial protocol, the existing body of safety and efficacy data for the product candidate, the number and nature of competing treatments and ongoing clinical trials of or expanded access to competing therapies for the same indication, the proximity of patients to clinical sites, the eligibility criteria for the trial, the ability to adequately monitor patients during a trial, clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied, and the risk that patients will drop out of a trial before completing all site visits. There are limited patient pools from which to draw in order to complete our clinical trials in a timely and cost-effective manner, including due to the fact that the diseases we target are rare. Moreover, for example, the patient population for PBH may decrease due to the development of novel treatments for obesity, reducing the potential need for bariatric surgery. Furthermore, our efforts to build relationships with patient communities may not succeed, which could result in delays in patient enrollment in our clinical trials.

Any negative results we may report in clinical trials of AMX0035, avexitide or any future product candidate may also make it difficult or impossible to recruit and retain patients in other clinical trials of that same product candidate. For example, the PHOENIX trial did not meet its primary or secondary endpoints, which may discourage patients from participating in clinical trials of AMX0035 in other indications. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop AMX0035 in Wolfram syndrome, PSP and additional indications, avexitide and any other current or future product candidates, or could render further development impossible. Further, if patients drop out of our clinical trials, miss scheduled doses or follow-up visits, or otherwise fail to follow clinical trial protocols, whether as a result of public health epidemics and related illness, the integrity of data from our clinical trials may be compromised or not accepted by the FDA or other regulatory authorities, which would represent a significant setback for the applicable program. In addition, we may rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance.

Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.

As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, it is common that various aspects of the development activities, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and results. Any of these changes could cause AMX0035, avexitide or any other current or future product candidates to perform differently and affect the results of ongoing clinical trials or other future clinical trials conducted with the materials manufactured using altered processes. For example, in seeking approval of AMX0035 in Europe, we submitted data supporting a different formulation of AMX0035 from the formulation evaluated in the CENTAUR trial. Changes to commercial formulations from those studied clinically could lead regulatory authorities to delay the approval of our marketing applications until we can demonstrate through additional clinical data that there is comparability in the bioavailability of the two different formulations or may require us to revert to the prior formulation evaluated clinically. Should we have to conduct comparability testing to bridge earlier clinical data obtained from product candidates produced under earlier manufacturing methods or formulations with the planned commercial formulation, regulatory authorities may disagree on the interpretation of results from this testing. This could delay completion of clinical trials, require the repetition of one or more clinical trials, increase clinical trial costs, delay approval of AMX0035, avexitide or any other current or future product candidates and jeopardize our ability to commence sales and generate revenue.

Certain of our product candidates require specific shipping, storage, handling and administration, which in some cases, may require cold-chain logistics and subject our product candidates to risk of loss or damage if failures occur.

Certain of our product candidates are sensitive to temperature, storage and handling conditions. They must be stored at very low temperatures in specialized freezers or specialized shipping containers until immediately prior to use. The handling and administration of the product, if approved, may need to be performed according to specific instructions and in some steps within specific time periods. Failure to correctly handle our product could negatively impact the efficacy and or safety of our product, or cause a loss of product. In addition, if approved, certain of our products may need to be frozen using specialized equipment and maintained following specific procedures in order to be stored without damage in a cost-efficient manner and without degradation. We will need to scale-up a cost-effective and reliable cold-chain distribution and logistics network, which we may be unable to accomplish. Failure to effectively scale-up our cold-chain supply logistics, by us or third parties, could in the future lead to additional manufacturing costs and delays in our ability to supply required quantities for commercial supply. For these and other reasons, we may not be able to manufacture our current or future product candidates at commercial scale or in a cost-effective manner. Even if we are able to manufacture and distribute the product candidates, if our products require specific procedures to maintain and use them, we may be limited in commercial opportunity.

AMX0035, avexitide or any future product candidate may cause undesirable side effects or have other properties that could delay or prevent its regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following regulatory approval, if obtained.

Undesirable side effects caused by AMX0035, avexitide or any future product candidate, could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay, denial or withdrawal of regulatory approval by the FDA or comparable foreign regulatory authorities. Results of our preclinical studies or clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. In clinical trials of AMX0035 to date, AMX0035 has been generally well-tolerated, with most common treatment-emergent adverse events including diarrhea, abdominal pain, nausea, upper respiratory infection, constipation, headache, fatigue, proteinuria, and decreased appetite. In addition, it has been reported that patients experience a bad taste when taking AMX0035. In animal studies, administration of AMX0035 to rats throughout pregnancy and lactation resulted in increased offspring mortality at all doses tested, which were less than or similar to the clinical doses tested in our clinical trials. However, there can be no guarantee that we would observe a similar tolerability profile of AMX0035 in future clinical trials or for other indications. Many compounds that initially showed promise in clinical or earlier stage testing are later found to cause undesirable or unexpected side effects that prevented further development of the compound.

In clinical trials to date of avexitide, avexitide was generally well-tolerated. The most common adverse events were injection site bruising, headache, and nausea; these occurred more often with placebo than either avexitide dose. However, there can be no guarantee that we would observe a similar tolerability profile of avexitide in future clinical trials or for other indications.

If unacceptable or severe side effects arise in the development of AMX0035, avexitide or any other current or future product candidates, we, the FDA or comparable foreign regulatory authorities, the IRBs, or independent ethics committees at the institutions in which our trials are conducted, or the independent safety monitoring committee could suspend or terminate our clinical trials or regulatory authorities could order us to cease clinical trials or deny approval of AMX0035, avexitide or any other current or future product candidates for any or all targeted indications. Treatment-emergent side effects that are deemed to be drug-related could also affect subject recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. Undesirable side effects in one of our clinical trials for AMX0035 in one indication or avexitide could adversely affect enrollment in clinical trials, regulatory approval and commercialization of AMX0035 in other indications or avexitide. Additionally, there may be negative findings regarding components of AMX0035, avexitide or future product candidates by other parties. Any negative findings by third parties may impact the future approvability or labeling of AMX0035, avexitide or other product candidates we may develop. In addition, side effects may not be appropriately recognized or managed by the treating medical staff. Inadequate training in recognizing or managing the potential side effects of AMX0035, avexitide or any future product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

Bitter taste was frequently observed in our clinical trials of AMX0035. While bitter taste, by itself, does not present a safety risk for patients, it may lead to higher levels of patient non-compliance, which could have the effect of reducing the observed efficacy of AMX0035 in our clinical trials, or limit its commercial adoption. AMX0035 is a combination of TURSO and PB. PB has been approved by the FDA and other regulatory authorities for the treatment of patients with certain urea cycle disorders and TURSO has been approved in Italy for diseases of cirrhotic liver disorders such as primary biliary cirrhosis. It is possible that one or more of the active moieties in AMX0035 has also been approved by FDA or other regulatory authorities. Even if AMX0035 receives marketing approval and is commercialized in a jurisdiction, we would continue to be subject to the risks that the applicable regulatory authorities could revoke approval of PB or TURSO or any active moiety in AMX0035, if applicable, or that efficacy, manufacturing or supply issues could arise with PB or TURSO or any active moiety in AMX0035, if applicable. This could result in our own products being removed from the market or being less commercially successful.

Finally, clinical trials of AMX0035 and avexitide are conducted in carefully defined sets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of any future collaborator, may indicate an apparent positive effect of AMX0035, avexitide or any other current or future product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects.

Demand for expanded access to AMX0035 could negatively affect our reputation and harm our business.

We are developing AMX0035 for the treatment of Wolfram syndrome, PSP and other potential future indications for which there are currently limited or no available therapeutic options. It is possible for individuals or groups to target companies with disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide access to AMX0035 or any of our future product candidates under an expanded access policy, our reputation may be negatively affected and our business may be harmed.

In the past, media attention to individual patients' expanded access requests has resulted in the introduction and enactment of legislation at the local and national level, including "Right to Try" laws, such as the federal Right to Try Act of 2017, which are intended to allow patients access to unapproved therapies earlier than traditional EAPs. A possible consequence of both activism and legislation in this area may be the need for us to initiate an EAP beyond that which we have submitted to the FDA or to make AMX0035 or any future product candidates more widely available sooner than anticipated.

In addition, some patients who receive access to drugs prior to their commercial approval through compassionate use, EAPs or right to try access have life-threatening illnesses and have exhausted all other available therapies. The risk for SAEs in this patient population is high, which could have a negative impact on the safety profile of AMX0035 or future product candidates, which could cause significant delays or an inability to successfully commercialize AMX0035 or future product candidates, which could materially harm our business. We may in the future need to restructure or pause any future compassionate use and/or EAP we initiate in order to perform the controlled clinical trials required for regulatory approval and successful commercialization of AMX0035 or future product candidates, which could prompt adverse publicity or other disruptions related to current or potential participants in such programs.

If we fail to develop and commercialize AMX0035 for additional indications or avexitide or fail to discover, develop or acquire and commercialize other product candidates, we may be unable to grow our business and our ability to achieve our strategic objectives would be impaired.

We are currently, and plan to continue to, evaluate AMX0035 in other indications other than ALS, to continue to develop and evaluate avexitide and develop other product candidates. We intend to evaluate internal opportunities from AMX0035, avexitide or other potential product candidates, and also may choose to in-license or acquire other product candidates as well as commercial products to treat patients suffering from neurodegenerative diseases and CNS or other disorders with significant unmet medical needs and limited treatment options. For example, we recently completed the acquisition of substantially all of the rights, title and interests in, to and under those assets and interests used by Eiger in the development, manufacture and commercialization of avexitide. Avexitide has been evaluated in five Phase 2 clinical studies for PBH and Congenital HI, both diseases with unmet need, and we intend to initiate a Phase 3 program in PBH.

Avexitide and any other potential product candidates have and will require additional, time-consuming development efforts prior to commercial sale, including preclinical studies, clinical trials and approval by the FDA and/or other applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, we cannot assure you that any such products that are approved will be manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives.

Research activities to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research activities or observation of third-party research activities may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete;
- product candidates that we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

If we are unsuccessful in identifying and developing additional product candidates, our potential for growth and achieving our strategic objectives may be impaired.

We may not be successful in our efforts to expand our pipeline by identifying additional product candidates or indications and modifications for which to investigate AMX0035 or avexitide in the future. We may expend our limited resources to pursue particular product candidates or indications or formulations for AMX0035 or avexitide and fail to capitalize on such product candidates or indications or formulations of AMX0035 or avexitide that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we are focused on specific indications and modifications for AMX0035, avexitide and AMX0114. As a result, we may fail to generate additional clinical development opportunities for such candidates for a number of reasons, including, that such candidates may in certain indications, on further study, be shown to have harmful side effects, limited to no efficacy, or other characteristics that suggest it is unlikely to receive marketing approval and achieve market acceptance in such additional indications.

We plan to conduct several clinical trials for AMX0035 in parallel over the next several years, including clinical trials in patients with Wolfram syndrome, PSP and other indications, which may make our decision as to which indication to prioritize more difficult. Moreover, we intend to conduct a clinical trial of avexitide in PBH and may conduct others in other indications as well. As a result, we may forgo or delay pursuit of opportunities with other indications that we believe could have had greater commercial potential or likelihood of success. In addition, we are continuing to evaluate plans to explore the use of AMX0035 in patients with AD, and other product candidates in ALS and additional neurodegenerative diseases. However, we may focus on or pursue one or more of our target indications over other potential indications and product candidates and such development efforts may not be successful, which would cause us to delay the clinical development and approval of AMX0035, avexitide, and other product candidates. Furthermore, research activities to identify additional indications for AMX0035, avexitide and other product candidates require substantial technical, financial, and human resources. We may not successfully develop these additional modifications for chemistry-related, stability-related, or other reasons. We have recently announced the development of AMX0114, an antisense oligonucleotide, targeting Calpain-2 for ALS and other neurodegenerative diseases. We are currently advancing AMX0114 through IND-enabling studies and expect to enter the clinic in 2024. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development activities for specific indications or formulations of AMX0035 or for AMX0114 or other product candidates may not yield any commercially viable products.

Additionally, we may pursue in-licenses or acquisitions of development-stage assets or programs, which entails additional risk to us. For example, we recently completed the acquisition of substantially all of the rights, title and interests in, to and under those assets and interests used by Eiger in the development, manufacture and commercialization of avexitide. Avexitide has been evaluated in five Phase 2 clinical studies for PBH and congenital HI, both diseases with unmet need, and we intend to initiate a Phase 3 program in PBH. Identifying, selecting and acquiring promising product candidates requires substantial technical, financial, and human resources expertise and, once acquired, requires us to devote substantial resources. Efforts to do so may not result in the actual acquisition or license of a particular product candidate, and, if acquired, may results in extensive diligence and preparation efforts, each of which may potentially result in a diversion of our management's time and the expenditure of our resources with no resulting benefit.

For example, if we are unable to identify programs that ultimately result in approved products, we may spend material amounts of our capital and other resources evaluating, acquiring and developing products that ultimately do not provide a return on our investment.

Competitive products may reduce or eliminate the commercial opportunity for AMX0035 for our intended indications or avexitide. If our competitors develop technologies or product candidates more rapidly than we do, or their technologies or product candidates are more effective or safer than ours, our ability to develop and successfully commercialize AMX0035 or avexitide may be adversely affected.

The clinical and commercial landscape for the treatment of the diseases we are focused on is highly competitive and subject to rapid and significant technological change. We will face competition with respect to any future indications of AMX0035, avexitide or other candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are also a number of large pharmaceutical and biotechnology companies that currently market and sell drugs or are pursuing the development of drug candidates for the treatment of the indications that we are pursuing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Many of our competitors have significantly greater financial resources, established presence in the market, expertise in research and development, manufacturing, preclinical and clinical testing, obtaining regulatory approvals and reimbursement and marketing approved products than we do. Accordingly, our competitors may be more successful than we may be in obtaining regulatory approval for therapies and achieving widespread market acceptance. Our competitors' products may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render AMX0035, avexitide or any future product

candidates obsolete or non-competitive before we can recover development and commercialization expenses. If AMX0035 or avexitide is approved for the indications we are currently pursuing, it could compete with a range of therapeutic treatments that are in development. In addition, our competitors may succeed in developing, acquiring or licensing technologies and products that are more effective or less costly than AMX0035, avexitide or any future product candidates that we may develop, which could render such product candidates obsolete and noncompetitive.

Following any approval for AMX0035, avexitide or any other future product candidate, we may face competition based on many different factors, including the efficacy, safety and tolerability of our products, the ease with which our products can be administered, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Existing and future competing products could present superior treatment alternatives, including being more effective, safer, less expensive or marketed and sold more effectively than any products we commercialize. Competitive products may make any products we commercialize obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. Such competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute our business plan.

In addition, our competitors may obtain patent protection, regulatory exclusivities or regulatory approval and commercialize products more rapidly than we do, which may impact future approvals or sales of any of our product candidates that receive regulatory approval. Following approval by the FDA or other foreign regulatory bodies for the commercial sale of AMX0035, avexitide or any future product candidates, we will also be competing with respect to marketing capabilities and manufacturing efficiency. We expect competition among products will be based on product efficacy and safety, the timing and scope of regulatory approvals, availability of supply, marketing and sales capabilities, product price, reimbursement coverage by government and private third-party payors, regulatory exclusivities and patent position. Our profitability and financial position will suffer if our product candidates receive regulatory approval, but cannot compete effectively in the marketplace.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites, as well as in acquiring technologies complementary to, or necessary for, our activities.

Obtaining and maintaining regulatory approval of AMX0035, avexitide or any future product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of those product candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of AMX0035, avexitide and any future product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S. and other jurisdictions, including additional preclinical studies or clinical trials, as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the U.S., including Canada, and certain jurisdictions in the EU, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

Regulatory authorities in jurisdictions outside of the U.S. have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions and such regulatory requirements can vary widely from country to country. Obtaining other regulatory approvals and compliance with other regulatory requirements could result in significant delays, difficulties and costs for us and could require additional preclinical studies or clinical trials, which could be costly and time-consuming and could delay or prevent the introduction of our products in certain countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. We do not have experience in obtaining regulatory approval in international markets outside of Canada. If we fail to comply with the regulatory requirements in international markets and/or obtain and maintain applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of AMX0035, avexitide or any future product candidates will be harmed.

Even though we have obtained orphan drug designation for AMX0035 for the treatment of Wolfram syndrome and for avexitide for the treatment of hyperinsulinemic hypoglycemia (which includes PBH and Congenital HI) in the U.S. and for Congenital HI in Europe by the EMA, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.

Regulatory authorities in some jurisdictions, including the U.S. and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a

rare disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the U.S., or a patient population of greater than 200,000 people in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the EU, an orphan designation may be granted in respect of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than five in 10,000 people in the EU when the application is made. Additionally, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition when, without incentives, it is unlikely that sales of the product in the EU would be sufficient to justify the necessary investment in developing the product. In either case, the applicant for orphan designation must also demonstrate that no satisfactory method of diagnosis, prevention, or treatment for the condition has been authorized (or, if such a method exists, the new product would be a significant benefit to those affected compared to the product available).

We received orphan drug status for AMX0035 for the treatment of patients with Wolfram syndrome in the U.S. in November 2020. Eiger received orphan drug status for avexitide for the treatment of hyperinsulinemic hypoglycemia (which includes PBH and Congenital HI) in the U.S. in December 2016 and for Congenital HI in Europe by the EMA in November 2019. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the other regulatory bodies from approving another marketing authorization application for the same drug for that time period. Another drug may receive marketing approval prior to AMX0035 or avexitide. The applicable period is seven years in the U.S. and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory agency's agreed upon pediatric investigation plan. The exclusivity period in the EU may be reduced to six years if, at the end of the fifth year, it is demonstrated that a product no longer meets the criteria for orphan designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. In the EU, during the ten-year period of orphan marketing exclusivity, neither the competent authorities of the EU Member States, the EMA, or the European Commission are permitted to accept applications or grant marketing authorization for similar medicinal products to the authorized orphan product. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. Legislation has been proposed by the European Commission that, if implemented, has the potential in some cases to shorten the ten-year period of orphan marketing exclusivity. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA and the EMA can subsequently approve another drug for the same condition before the expiration of the seven-year (or ten-year in the EU) exclusivity period if the FDA or the EMA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In addition, if an orphan designated product receives marketing approval for an indication broader than or different from what is designated, such product may not be entitled to orphan exclusivity. Even though the FDA has granted orphan drug designation to AMX0035 for the treatment of Wolfram syndrome, if we receive approval for AMX0035 for a modified or different indication, our current orphan designation may not provide us with exclusivity.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process. Also, regulatory approval for any product candidate may be withdrawn, and other product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

Even if we obtain orphan drug exclusivity for AMX0035 or avexitide, that exclusivity may not effectively protect us from competition because different drugs can be approved for the same condition before the expiration of the orphan drug exclusivity period. For example, even though AMX0035 is entitled to orphan drug exclusivity, that exclusivity may not prevent the approval of TURSO by the FDA or other regulatory authorities as a monotherapy treatment for Wolfram syndrome if those regulatory agencies determine that TURSO is a different drug product from AMX0035. In addition, the regulatory authorities may find that this monotherapy treatment is clinically superior to our fixed dose product and approve it even if we are granted orphan drug exclusivity. U.S. lawmakers have also recently raised the possibility that regulatory or legislative changes might need to be made to the Orphan Drug Act to foster competition. This includes the introduction of legislation that, if adopted into law, would require us to demonstrate to the FDA that AMX0035 or avexitide would be economically unviable when facing competition to maintain our exclusivity.

We may pursue orphan drug designation for AMX0035 or avexitide for the treatment of additional indications. The incidence and prevalence of the target patient populations for these indications will be based on our estimates and third-party data. If the market opportunity for these target populations is larger than we estimate, we may be unable to receive orphan drug designation. Additionally, if orphan drug designation is granted, we may be unable to maintain any benefits associated with orphan drug designation, including market exclusivity.

Periodically, we make estimates regarding the incidence and prevalence of target patient populations based on various third-party sources and internally generated analysis. Our estimates may be inaccurate or based on imprecise data. As described above, under the

Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the U.S., or a patient population of greater than 200,000 people in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. If our incidence or prevalence estimates for future indications for which we may seek orphan drug designation are incorrect, we may be unable to receive orphan drug designation.

Even if the FDA grants orphan drug designation for AMX0035 or avexitide for other indications, exclusive marketing rights in the U.S. may be limited if we seek FDA marketing approval for an indication broader than the orphan designated indication. Additionally, any product candidate that initially receives orphan drug status designation, may lose such designation if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In addition, others may obtain orphan drug status for products addressing the same diseases or conditions as products we are developing, thus limiting our ability to compete in the markets addressing such diseases or conditions for a significant period of time. As a result, our business and prospects could suffer.

We may pursue Priority Review Designation for product candidates that we may develop, but we might not receive such designations, and Priority Review Designations may not lead to a faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A Priority Review Designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. For example, we received priority review for AMX0035 for the treatment of ALS, and we may in the future request Priority Review Designation for any future product candidates, however, we cannot assume that any application for future indications of AMX0035, avexitide or any other product candidate we may develop will meet the criteria for that designation. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a Priority Review Designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to standard FDA review and approval. For example, the FDA originally set the PDUFA date for AMX0035 for the treatment of ALS, for June 29, 2022, and then extended the review timeline for our NDA to September 2022. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

We may seek Fast Track Designation by the FDA for a product candidate that we develop, and we may be unsuccessful. If we are successful, the designation may not actually lead to a faster development or regulatory review or approval process.

We may seek Fast Track Designation for future product candidates we develop. If a product is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address an unmet medical need for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may rescind the Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development activities.

Avexitide has been granted Breakthrough Therapy Designation for PBH and Congenital HI and we may seek Breakthrough Therapy Designation by the FDA for additional product candidates that we may develop, and we may be unsuccessful. If we are successful, the designation may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval, nor does such designation for avexitide guarantee a faster review process or marketing approval.

Avexitide has been granted Breakthrough Therapy Designation for PBH and Congenital HI and we may seek Breakthrough Therapy Designation for any additional product candidate that we may develop. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for accelerated approval and priority review.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe a product candidate we develop meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of Breakthrough Therapy Designation for a product candidate, such as avexitide, may not result

in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if any product candidate we develop qualifies as a breakthrough therapy, the FDA may later decide that the drug no longer meets the conditions for qualification and rescind the designation.

The FDA has granted rare pediatric disease designation to avexitide for the treatment of Congenital HI. However, a marketing application for avexitide or any other product candidate, if approved, may not meet the eligibility criteria for a priority review voucher.

The FDA has granted rare pediatric disease designation to avexitide for the treatment of Congenital HI. Designation of a drug as a drug for a rare pediatric disease does not guarantee that an NDA for such drug will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the FDCA, we will need to request a rare pediatric disease priority review voucher in our original NDA for avexitide. The FDA may determine that an NDA for avexitide, if approved, does not meet the eligibility criteria for a priority review voucher, including for the following reasons:

- Congenital HI no longer meets the definition of a rare pediatric disease;
- the NDA contains an active ingredient that has been previously approved by the FDA;
- the NDA does not rely on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population (that is, if the NDA does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or
- the NDA is approved for a different adult indication than the rare pediatric disease for which avexitide is designated.

The authority for the FDA to award rare pediatric disease priority review vouchers for drugs and biologics is currently limited to those candidates that receive rare pediatric disease designation on or prior to December 20, 2024, and the FDA may only award rare pediatric disease priority review vouchers through September 30, 2026. However, it is possible the FDA's authority to award rare pediatric disease priority review vouchers will be further extended by Congress as reauthorization legislation is currently pending. Absent any such extension, if an NDA for avexitide is not approved prior to September 30, 2026 for any reason, regardless of whether it meets the criteria for a rare pediatric disease priority review voucher, it will not be eligible for a priority review voucher.

Product liability lawsuits against us or any of our future collaborators could divert our resources and attention, cause us to incur substantial liabilities and limit commercialization of AMX0035, avexitide or any other current or future product candidates.

We are exposed to potential product liability and professional indemnity risks that are inherent in the research, development, manufacturing, marketing and use of pharmaceutical products. The use of AMX0035, avexitide or any other current or future product candidates by us and any collaborators in clinical trials, and the prior sales of AMX0035 in the U.S. and Canada and continued use pursuant to the free drug program may expose us to liability claims. Product liability claims may be brought against us or our partners by participants enrolled in our clinical trials, patients, health care providers, pharmaceutical companies, our collaborators or others using, administering or selling any of our future approved products. If we cannot successfully defend ourselves against any such claims, we may incur substantial liabilities or be required to limit commercialization of AMX0035, avexitide or any other current or future product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for any of our future approved products;
- injury to our reputation;
- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- significant litigation costs, including with respect to potential class action lawsuits;
- substantial monetary awards to, or costly settlements with, patients or other claimants;
- product recalls or a change in the indications for which they may be used;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize AMX0035, avexitide or any other current or future product candidates.

Although the clinical trial process is designed to identify and assess potential side effects, clinical development does not always fully characterize the safety and efficacy profile of a new drug, and it is always possible that a drug, even after regulatory approval, may

exhibit unforeseen side effects. If AMX0035, avexitide or any other current or future product candidates was to cause adverse side effects during clinical trials or after approval, we may be exposed to substantial liabilities. Physicians and patients may not comply with any warnings that identify known potential adverse effects and patients who should not use AMX0035, avexitide or any of our future product candidates. If any of our current or future product candidates are approved for commercial sale, we will be highly dependent upon consumer perceptions of us and the safety and quality of our products. We could be adversely affected if we are subject to negative publicity associated with illness or other adverse effects resulting from patients' use or misuse of our products or any similar products distributed by other companies.

Although we maintain product liability insurance coverage in the amount of up to $10.0 million in the aggregate, including clinical trial liability, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We may need to increase our insurance coverage as we commercialize AMX0035 or avexitide in the U.S. and other jurisdictions, if approved, or any other current or future product candidate that receives regulatory approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of AMX0035, avexitide or any other current or future product candidates, which could harm our business, financial condition, results of operations and prospects.

Even if we, or any future collaborators, obtain and maintain regulatory approvals for AMX0035, avexitide or any other current or future product candidate, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products, which could impair our ability to generate revenue.

Once regulatory approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and any future collaborators, must therefore comply with requirements concerning advertising and promotion for AMX0035, avexitide or any other current or future product candidate for which we or they obtain regulatory approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and any future collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA and other regulatory bodies' requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, any future collaborators and their contract manufacturers could fail to conform to cGMPs and be subject to periodic unannounced inspections by the FDA and other regulatory bodies to monitor and ensure compliance with cGMPs. Despite our efforts to inspect and verify regulatory compliance, one or more of our third-party manufacturing vendors may be found on regulatory inspection by the FDA or other authorities to be not in compliance with cGMP regulations, which may result in shutdown of the third-party vendor or invalidation of drug product lots or processes. In some cases, a product recall may be warranted or required, which would materially affect our ability to supply and market our drug products.

Accordingly, in any jurisdiction where we or any future collaborators, receive regulatory approval for AMX0035, avexitide or one or more future product candidates, we, and any future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and any future collaborators, are not able to comply with post-approval regulatory requirements, we, and any future collaborators, could have the regulatory approvals for AMX0035, avexitide or any other current or future products withdrawn by regulatory authorities and our, or any future collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Laws and regulations governing any international operations we expect to have in the future may preclude us from developing, manufacturing and selling certain products outside of the U.S. and will require us to develop and implement costly compliance programs.

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the U.S. to comply with certain accounting provisions requiring us to maintain

books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders, including export control and trade sanctions laws, also restrict the use and dissemination outside of the U.S., or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the U.S., it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the U.S., which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Risks Related to Our Dependence on Third Parties

We may seek to establish collaborations and if we are not able to establish and maintain them on commercially reasonable terms, we may have to alter our development and future commercialization plans.

The advancement of AMX0035, avexitide, and any other current or future product candidates and development programs or activities, will require substantial additional cash to fund expenses. For some indications of AMX0035, avexitide, or other current or future product candidates, we may decide to collaborate with additional pharmaceutical and biotechnology companies with respect to development and potential commercialization. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. In addition, if we are able to obtain regulatory approval for product candidates from foreign regulatory authorities, we may enter into collaborations with international biotechnology or pharmaceutical companies for the commercialization of such product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA or other comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the

potential of competing products. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available for collaboration and whether such a collaboration could be more attractive than the one with us for our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop AMX0035, avexitide, or any other current or future product candidates or bring them to market and generate product revenue.

Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Any collaboration agreements that we enter into in the future may contain restrictions on our ability to enter into potential collaborations or to otherwise develop specified product candidates. We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs or activities, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or future commercialization activities at our own expense.

We have entered and may in the future enter into collaborations with third parties for the development and commercialization of AMX0035, avexitide or any other current or future product candidates, and our prospects with respect to AMX0035, avexitide and our other current or future product candidates will depend in significant part on the success of those collaborations.

We may rely on collaborations for the development and future commercialization of AMX0035, avexitide and any other current or future product candidates. For example, we may utilize a variety of distribution, collaboration and other marketing arrangements with one or more third parties to facilitate commercialization of AMX0035 or avexitide or to identify novel drug candidates for neurodegenerative or other diseases. Our likely collaborators for any distribution, development, sales, marketing, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into such collaborations, we may have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of AMX0035, avexitide or any other current or future product candidates. Our ability to generate revenues from these arrangements will depend on any future collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving AMX0035, avexitide and any other current or future product candidates pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of AMX0035, avexitide or any future product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with AMX0035, avexitide or any of our other current or future product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, including trade secrets and intellectual property rights, contract interpretation, or the preferred course of development might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;

- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours is involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

We rely on third parties to assist in conducting our clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or successfully commercialize AMX0035, avexitide or any other current or future product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.

We have relied upon and plan to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials and expect to rely on these third parties to conduct clinical trials of any future product candidate that we develop. Any of these third parties may terminate their engagements with us under certain circumstances. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new CRO begins work. As a result, delays may occur, which could negatively impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects. Clinical trials involve multiple clinical sites, vendors and other third parties and we are dependent on these vendors to ensure appropriate study conduct, statistical analysis and randomization. Errors or deviations they make in any of these activities could impact the usefulness and interpretability of clinical trial results by regulatory authorities. For example, at the Advisory Committee meeting on March 30, 2022, the FDA noted a number of concerns that, in the FDA's view, impacted the interpretability of the results from the CENTAUR trial. Clinical trials from time to time have deviations where a protocol or standard operating procedure is not perfectly carried out and where corrective actions are taken. While we may perceive these events as low risk, our perception of risk and appropriate corrective actions may differ from that of the regulators' view. Deviations from protocols or standard operating procedures during studies could result in negative regulatory opinions and outcomes.

Further, although our reliance on these third parties for clinical development activities limits our control over these activities, we remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. Moreover, the FDA and competent authorities of the EU Member States require us to comply with Good Clinical Practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and IRBs. If we or our third-party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or other regulatory body may require us to perform additional clinical trials before approving AMX0035 or avexitide, including for additional indications, or any other current or future product candidates, which would delay the regulatory approval process. We cannot be certain that, upon inspection, the FDA or other regulatory body will determine that any of our clinical trials comply with GCPs. For example, our clinical trial sites and investigators have in the past and may in the future engage in protocol deviations which could impact the overall interpretability of the outcomes of our clinical trials. We are also required to register certain clinical trials and post the results of completed clinical trials on a government-sponsored database, such as ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, the third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development activities. These contractors may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could impede their ability to devote appropriate time to our clinical activities. If these third parties, including clinical investigators, do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, clinical data necessary for regulatory approvals for AMX0035, avexitide or any other current or future product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize AMX0035, avexitide or any other current or future product candidates. In such an event, our financial results and the commercial prospects for AMX0035, avexitide or any other current or future product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or regulatory approval of AMX0035, avexitide or any other current or future product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue.

Our use of third parties to manufacture AMX0035 or avexitide in compliance with cGMP may increase the risk that we will not have sufficient cGMP-compliant quantities of AMX0035 or avexitide or necessary quantities of such materials on time or at an acceptable cost.

We do not own or operate manufacturing facilities for the production of clinical or commercial quantities of AMX0035 or avexitide, and we currently lack the resources and the capabilities to do so. As a result, we currently rely on third parties for the manufacture and supply of the active pharmaceutical ingredients, or APIs, in AMX0035 and avexitide, and for the blending and packaging of AMX0035 and avexitide in accordance with applicable law, regulations and standards. Our current strategy is to outsource all manufacturing of AMX0035 and avexitide and any other current or future product candidates to third parties.

We currently engage third-party manufacturers to provide the APIs of AMX0035 and avexitide and for the final drug product formulation of AMX0035 and avexitide that is or will be being used in our clinical trials and for expanded access and commercial supply, as applicable (currently being supplied without cost to patients who remained on RELYVRIO or ALBRIOZA after April 4, 2024), and we engage separate third-parties for the blending and packaging of finished clinical materials. We must be able to demonstrate comparability of drug substance across suppliers along with stability data across suppliers. We currently rely on a single manufacturer to supply one of our APIs and a separate manufacturer to supply the other. Although we believe that there are several potential alternative manufacturers who could manufacture each of the APIs in AMX0035 and avexitide, we may incur added costs and delays in identifying and qualifying any such replacement. Moreover, the extent to which geopolitical events or global health crises may impact our ability to procure sufficient supplies for the development of AMX0035 and avexitide, and any other current or future products and product candidates will depend on whether the economic challenges caused by such events continue to impact the global economy and supply chains, among many other factors. There is no assurance that we will be able to timely secure needed supply arrangements on satisfactory terms, or at all. Our failure to secure these arrangements as needed could have a material adverse effect on our ability to complete the development of AMX0035, avexitide or any other current or future product candidates or, to commercialize them, if approved. We may be unable to conclude agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms. There may be difficulties in scaling up to commercial quantities and formulation of AMX0035 and avexitide, and the costs of manufacturing could be prohibitive.

Following our announcement to begin the process to voluntarily withdraw RELYVRIO and ALBRIOZA from the market, we entered into negotiations with each of our third-party manufacturers to redefine our relationship going forward. These negotiations are ongoing and may result in irreparable damage to our relationship with one or more suppliers, making our further development and potential commercialization challenging.

Even if we are able to establish and maintain arrangements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the failure of the third-party manufacturer to comply with applicable regulatory requirements, including cGMPs, and reliance on third-parties for manufacturing process development, regulatory compliance and quality assurance;
- manufacturing delays if our third-party manufacturers give greater priority to the supply of other products over AMX0035, avexitide or any other current or future product candidates or otherwise do not satisfactorily perform according to the terms of the agreement between us;
- limitations on supply availability resulting from capacity and scheduling constraints of third-parties, or as a result of economic or political developments, including the ongoing conflicts in Ukraine and Israel and global economic instability;
- the possible breach of manufacturing agreements by third-parties because of factors beyond our control;
- the possible termination or non-renewal of the manufacturing agreements by a third-party, at a time that is costly or inconvenient to us; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

If we do not maintain our key manufacturing relationships, or if any of our contract manufacturers fail to perform their obligations, we may fail to find replacement manufacturers or develop our own manufacturing capabilities, which could delay or impair our ability to obtain regulatory approval for our products. If we do find replacement manufacturers, we may not be able to enter into agreements with them on terms and conditions favorable to us and there could be a substantial delay before new facilities could be qualified and registered with the FDA and other foreign regulatory authorities.

Any change in manufacturer may also involve changes in manufacturing procedures and processes, which could require that we conduct bridging studies between our prior clinical supply used in our clinical trials and that of any new manufacturer. In addition, we will need to verify that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA or another regulatory authority. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials.

In some cases, the technical skills required to manufacture AMX0035, avexitide, or any other current or future products or product candidates may be unique or proprietary to the original contract manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. Furthermore, a contract manufacturer may possess or acquire technology related to the manufacture of AMX0035, avexitide or any other current or future product candidate that such contract manufacturer owns independently. This would increase our reliance on such contract manufacturer or require us to obtain a license from such contract manufacturer in order to have another contract manufacturer manufacture AMX0035, avexitide or any other current or future product candidates. If AMX0035 for any of our initial or potential additional indications, avexitide or any future product candidate is approved by any regulatory agency, we intend to utilize arrangements with third-party contract manufacturers for the commercial production of those products. This process is difficult and time consuming and we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under cGMPs that are capable of manufacturing AMX0035, avexitide or any other current or future product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization.

Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, seizures or voluntary recalls of AMX0035, avexitide or any other current or future product candidates, operating restrictions and criminal prosecutions, any of which could significantly affect supplies of AMX0035, avexitide or any other current or future product candidates. The facilities used by our contract manufacturers to manufacture AMX0035, avexitide or any other current or future product candidates must be evaluated by the FDA and certain other foreign regulatory authorities. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with cGMPs. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, we may not be able to secure and/or maintain regulatory approval for our product manufactured at these facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or other comparable foreign regulatory authority finds deficiencies or does not approve these facilities for the manufacture of AMX0035, avexitide or any other current or future product candidates, or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market AMX0035, avexitide or any other current or future product candidates, if approved. Furthermore, if we are required to change contract manufacturers, we will be required to verify that the new contract manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations, which could result in further costs and delays. Contract manufacturers may face manufacturing or quality control problems causing drug substance production and shipment delays or a situation where the contractor may not be able to maintain compliance with the applicable cGMP requirements. Any failure to comply with cGMP requirements or other FDA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop AMX0035, avexitide or any other current or future product candidates and market our products, if approved.

The FDA and other foreign regulatory authorities require manufacturers to register manufacturing facilities. The FDA and corresponding foreign regulators also inspect these facilities to confirm compliance with cGMPs. Contract manufacturers may face manufacturing or quality control problems causing drug substance production and shipment delays or a situation where the contractor may not be able to maintain compliance with the applicable cGMP requirements. Any failure to comply with cGMP requirements or other FDA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop AMX0035, avexitide or any future product candidates and market our products following approval.

We may need to maintain licenses for active ingredients from third parties to develop and commercialize AMX0035, avexitide or a future product candidate, which could increase our development costs and delay our ability to commercialize such product candidate.

Should we decide to use API in any of AMX0035, avexitide or any other current or future product candidates that are proprietary to one or more third parties, we would need to maintain licenses to those active ingredients from those third parties. If we are unable to gain or continue to access rights to these active ingredients prior to conducting preclinical toxicology studies intended to support clinical trials, we may need to develop alternate product candidates from these programs by either accessing or developing alternate active ingredients, resulting in increased development costs and delays in commercialization of these product candidates. If we are

unable to gain or maintain continued access rights to the desired active ingredients on commercially reasonable terms or develop suitable alternate active ingredients, we may not be able to commercialize product candidates from these programs.

Risks Related to Commercialization of AMX0035, Avexitide or Future Product Candidates

The market for AMX0035 for Wolfram syndrome, PSP and other neurodegenerative diseases and of avexitide for PBH and congenital HI, and for any other product candidates we are currently developing or may in the future develop or acquire may be smaller than we expect.

We have historically focused our research and product development on treatments of neurodegenerative diseases, and recently expanded into other diseases, many of which are rare diseases with small addressable patient populations. We base our market opportunity estimates on a variety of factors, including our estimates of the number of people who have these diseases, the potential scope of our approved product labels, the subset of people with these diseases who have the potential to benefit from treatment with AMX0035, avexitide or any other current or future product candidates, various pricing scenarios, and our understanding of reimbursement policies for rare diseases in particular countries. These estimates are based on many assumptions and may prove incorrect, and new studies may reduce the estimated incidence or prevalence of these diseases. Estimating market opportunities can be particularly challenging for rare indications, such as the ones we currently address, as epidemiological data is often more limited than for more prevalent indications and can require additional assumptions to assess potential patient populations. If we are unable to identify patients and successfully commercialize AMX0035, avexitide or any other current or future product candidates with attractive market opportunities, our future product revenues may be smaller than anticipated, and our business may suffer.

Patient identification efforts also influence the ability to address a patient population. If efforts in patient identification are unsuccessful or less impactful than anticipated, for instance, because of a lack of diagnostic initiatives, inadequate disease awareness among healthcare professionals, difficulties in identifying and accessing patients outside of larger treatment centers or otherwise, we may not address the entirety of the opportunity we are seeking. As a result, patients may be difficult to identify and access, the addressable patient population in the countries in which we are seeking authorization and elsewhere may turn out to be lower than expected, or patients may not be otherwise amenable to treatment with our products, all of which would adversely affect our business, financial condition, results of operations and prospects.

If we are unable in the future to expand our sales, marketing, manufacturing and distribution capabilities or enter into agreements with third parties to market and sell AMX0035, avexitide or other current or future product candidates for which we obtain marketing approval, we will be unable to generate any additional product revenue.

To successfully commercialize any products that may result from our development activities or that we may acquire, we would need to continue to expand our sales, marketing, pharmacovigilance, manufacturing and distribution capabilities, either on our own or with others. RELYVRIO/ALBRIOZA, formerly sold in Canada and the U.S. for the treatment of ALS before being voluntarily withdrawn
from the market, was the first product that we commercialized and built a global marketing and sales team for. The development of our own marketing and distribution effort was expensive and time-consuming and any efforts to do so in connection with our other product candidates may be expensive and time-consuming and could delay any further product launches. Moreover, we cannot be certain that we will be able to develop this capability successfully again in the future, despite our experience. We may enter into collaborations regarding any approved product candidates with other entities to utilize their established marketing and distribution capabilities, however, we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize AMX0035, avexitide or any other current or future product candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We may also face competition in our search for third parties to assist us with the sales and marketing efforts of AMX0035, avexitide and any other current or future product candidates, if approved. Without an internal team or the support of a third-party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Even if any future product candidate of ours receives regulatory approval, it may fail to maintain the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for continued commercial success or to remain profitable.

Even if AMX0035 for the treatment of any indication, avexitide or any other current or future product candidate of ours, is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Physicians may be reluctant to add AMX0035, avexitide or another product to their patients' treatment regimen, or may cease to add AMX0035, avexitide or such product to their patients' treatment regimen. Further, patients often acclimate to the treatment regime they are currently taking and do not want to add additional treatments unless their physicians recommend it. Further, patients may be unable to add AMX0035, avexitide or such other product to their treatment regimen due to lack of coverage and adequate reimbursement. In addition, even if we are able to demonstrate our product candidates' safety and efficacy to the FDA and other regulators, safety or efficacy concerns in the medical community may

hinder market acceptance. Our ability to proactively educate health care professionals and patients may be limited based on the marketing restrictions in a given jurisdiction, specifically as they relate to the particular labeling approved by the applicable health authority.

Efforts to educate the medical community and third-party payors on the benefits of our current and any future product candidates may require significant resources, including management time and financial resources, and may not be successful. If AMX0035, avexitide or any other current or future product candidate is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not remain profitable. The degree of market acceptance of AMX0035, avexitide and any other future product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to competitive therapies and our ability to successfully publicize these advantages or highlight them in any marketing materials;
- the prevalence and severity of any side effects;
- whether the product is designated under physician treatment guidelines as a first-, second- or third-line therapy or as a single agent or in combination;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product's convenience, tolerability and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- limitations or warnings, including distribution or use restrictions contained in the product's approved labeling;
- the strength of sales, marketing and distribution support;
- changes in the standard of care for the targeted indications for the product; and
- availability and adequacy of coverage and reimbursement from government payors, managed care plans and other third-party payors.

Any failure by AMX0035, avexitide or any other current or future product candidate of ours that obtains regulatory approval to achieve market acceptance or commercial success would adversely affect our business prospects.

AMX0035, avexitide or any future product candidates for which we, or any future collaborators, obtain regulatory approval in the future will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. If approved, AMX0035, avexitide and any future product candidates could be subject to post-marketing restrictions or withdrawal from the market and we, or any future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

AMX0035, avexitide or any other current or future product candidates for which we, or any future collaborators, obtain regulatory approval, as well as the manufacturing processes, post-approval studies, labeling, advertising and promotional activities for such product, among other things, will be subject to ongoing requirements of and review by the FDA and other applicable regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. For certain commercial prescription drug products, manufacturers and other parties involved in the supply chain must also meet chain of distribution requirements and build electronic, interoperable systems for product tracking and tracing and notify the FDA of counterfeit, diverted, stolen and intentionally adulterated products or other products that are otherwise unfit for distribution in the U.S. We and our contract manufacturers will also be subject to user fees and periodic inspection by regulatory authorities to monitor compliance with these requirements and the terms of any product approval we may obtain. Even if regulatory approval of a product candidate is granted, the approval may be subject to limitations on the indications or uses for which the product may be marketed or to the conditions of approval, including the requirement in the U.S. to implement a Risk Evaluation and Mitigation Strategy, or REMS.

The FDA and other regulatory authorities may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. Additionally, the FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. Regulatory authorities impose stringent restrictions on manufacturers' communications regarding off-label use. However, companies generally

may share truthful and not misleading information that is otherwise consistent with a product's approved labeling. If we, or any future collaborators, do not market AMX0035, avexitide or any of our other current or future product candidates for which we, or they, receive regulatory approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing if it is alleged that we are doing so. Violation of laws and regulations relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws, including the False Claims Act and any comparable foreign laws. In the EU, the direct-to-consumer advertising of prescription-only medicinal products is prohibited. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public, if approved, and may also impose limitations on our promotional activities with health care professionals.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on the manufacturing of such products;
- restrictions on the labeling or marketing of such products;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- exclusion from federal health care programs such as Medicare and Medicaid;
- suspension or withdrawal of regulatory approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

If we are not able to comply with post-approval regulatory requirements, we could have the marketing approvals for AMX0035, avexitide or any future approved products withdrawn or restricted by regulatory authorities, or we may voluntarily do so, and our ability to market AMX0035, avexitide or any future approved products, to develop AMX0035 or avexitide in the U.S. or additional jurisdictions or for additional indications, and to develop and seek approval for additional product candidates could become limited, which could adversely affect our ability to achieve or sustain profitability. As a result, the cost of compliance with post-approval regulatory requirements may have a negative effect on our operating results and financial condition.

If we fail to obtain coverage and reimbursement for AMX0035, avexitide or any other current or future product candidates in new geographies, it could make it difficult for us to sell AMX0035, avexitide or any other current or future product candidates profitably.

The success of AMX0035, avexitide and any of our other current or future product candidates, if approved, depends on the availability of adequate coverage and reimbursement from third-party payors. Because AMX0035, avexitide and any other current or future product candidates represent new approaches to the treatment of the diseases they target, we cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, AMX0035, avexitide and any other current or future product candidates or for any product that we may develop. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any such product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to any current or future product candidates we may develop (e.g., for the administration of our product candidate to patients) is also important. Inadequate reimbursement for such services may lead to physician and payor resistance and adversely affect our ability to market or sell AMX0035, avexitide or any other current or future product candidates we may develop. In addition, we may need to develop new reimbursement models, in order to realize adequate value. Payors may not be

able or willing to adopt such new models and patients may be unable to afford that portion of the cost that such models may require them to bear. If we determine such new models are necessary, but we are unsuccessful in developing them, or if payors do not adopt such models, our business, financial condition, results of operations and prospects could be adversely affected. For additional information on coverage and reimbursement, see the section entitled "*Business—Government Regulation—Coverage and Reimbursement*" in our 2023 Annual Report.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors, such as private health insurers and health maintenance organizations, are critical to new product acceptance. Government authorities and other third-party payors decide which drugs and treatments they will cover and the reimbursement amount. Coverage and reimbursement by a third-party payor may depend upon a number of factors.

In the U.S., no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement from third-party payors will be obtained. There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products, which uncertainty may be heightened where the product is subject to post-marketing conditions or requirements to provide additional clinical data. In the U.S., the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Future coverage and reimbursement may be subject to increased restrictions, such as prior authorization requirements, both in the U.S. and in international markets. Orphan drugs are typically placed on the highest cost-sharing tier and a substantial percentage are subject to prior authorization requirements. Reimbursement agencies in the EU may be more conservative than CMS.

Outside the U.S., international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Canada, the EU and other countries has and will continue to put pressure on the pricing and usage of drug products such as AMX0035, avexitide and any other current or future product candidates we may develop, if approved. We may also incur additional challenges when seeking reimbursement from public and private payers where AMX0035, avexitide or any future product candidate has been approved subject to post-marketing conditions. Moreover, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product, possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the U.S. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the U.S., the reimbursement for AMX0035, avexitide and any other current or future product candidates we may develop may be reduced compared with the U.S. and may be insufficient to generate commercially reasonable revenues and profits.

Ongoing healthcare legislative and regulatory reform measures may have a material adverse effect on our business and results of operations.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business. For more information, see the section entitled "*Business – Government Regulation – Current and Future U.S. Healthcare Reform Legislation*" in our 2023 Annual Report.

We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for AMX0035, avexitide or any other current or future product candidates;
- our ability to set a price that we believe is fair for our approved products;
- our ability to generate revenue and achieve or maintain profitability;

- the level of taxes that we are required to pay; and
- the availability of capital.

These laws and future state and federal healthcare reform measures that may be adopted in the future may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for AMX0035, avexitide or any other current or future product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used. For example, the Inflation Reduction Act of 2022, or IRA, contains provisions that require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation. In addition, any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, sustain profitability or commercialize our product candidates.

Moreover, increasing efforts by governmental and third-party payors in the U.S. and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. There has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. The effect of these reform efforts on our business and the healthcare industry in general is not yet known.

Additional state and federal healthcare reform measures are expected to be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for certain pharmaceutical products or additional pricing pressures.

While some of these and other proposed measures may require additional authorization to become effective, Congress and the Biden administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs.

Governments outside the U.S. may impose strict price controls, which may adversely affect our revenues, if any.

In some countries, including Canada and certain Member States of the EU, the pricing of prescription drugs is, in part, subject to governmental control. Additional countries may adopt similar approaches to the pricing of prescription drugs. In such countries, pricing negotiations with governmental authorities can take considerable time after receipt of regulatory approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after coverage and reimbursement have been obtained. Reference pricing used by various countries and parallel distribution, or arbitrage between low-priced and high-priced countries, can further reduce prices. In some countries, we may be required to conduct a clinical trial or other trials that compare the cost-effectiveness of AMX0035, avexitide or any other current or future product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval, which is time-consuming and costly. We cannot be sure that such prices and reimbursement will be acceptable to us. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of AMX0035, avexitide or any other current or future product candidates in those countries would be negatively affected.

Our relationships with healthcare providers, physicians, patients and third-party payors may be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the U.S. and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, or FCA, which may constrain the business or financial arrangements and relationships through which such companies conduct research, sell, market and distribute pharmaceutical products. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the

improper use of information obtained in the course of patient recruitment for clinical trials. For more information, see the section entitled "*Business – Government Regulation - Other U.S. Healthcare Laws*" in our 2023 Annual Report.

In the U.S., to help patients afford our approved product, we offer programs to assist them or support third-party organizations' programs to assist patients, including patient assistance programs and co-pay coupon programs for eligible patients. Government enforcement agencies have shown increased interest in pharmaceutical companies' product and patient assistance programs, including reimbursement support services, and a number of investigations into these programs have resulted in significant civil and criminal settlements. In addition, at least one insurer has directed its network pharmacies to no longer accept co-pay coupons for certain specialty drugs the insurer identified. Our co-pay coupon programs could become the target of similar insurer actions. In September 2014, the HHS Office of Inspector General, or OIG, issued a Special Advisory Bulletin warning manufacturers that they may be subject to sanctions under the federal anti-kickback statute and/or civil monetary penalty laws if they do not take appropriate steps to exclude Part D beneficiaries from using co-pay coupons. Accordingly, companies exclude these Part D beneficiaries from using co-pay coupons. It is possible that changes in insurer policies regarding co-pay coupons and/or the introduction and enactment of new legislation or regulatory action could restrict or otherwise negatively affect these patient support programs, which could result in fewer patients using affected products, and therefore could have a material adverse effect on our sales, business, and financial condition.

Third party patient assistance programs that receive financial support from companies have also become the subject of enhanced government and regulatory scrutiny. The OIG has established guidelines that suggest that it is lawful for pharmaceutical manufacturers to make donations to charitable organizations who provide co-pay assistance to Medicare patients. However, donations to patient assistance programs have received some negative publicity and have been the subject of multiple government enforcement actions, related to allegations regarding their misuse to promote branded pharmaceutical products over other less costly alternatives. Specifically, in recent years, there have been multiple settlements resulting out of government claims challenging the legality of third party patient assistance programs under a variety of federal and state laws. We have in the past and may, from time to time, make charitable grants to independent charitable foundations that help financially needy patients with their premium, co-pay, and co-insurance obligations. If we choose to do so, and if we or our vendors or donation recipients are deemed to fail to comply with relevant laws, regulations or evolving government guidance in the provision of charitable donations or operation of these programs, we could be subject to damages, fines, penalties, or other criminal, civil, or administrative sanctions or enforcement actions. We cannot ensure that our compliance controls, policies, and procedures will be sufficient to protect against acts of our employees, business partners, vendors or charitable foundations that may violate the laws or regulations of the jurisdictions in which we operate. Regardless of whether we have complied with the law, a government investigation, including of any business partners, vendors or charitable foundations, could impact our business practices, harm our reputation, divert the attention of management, increase our expenses, and reduce the availability of foundation support for our patients who need assistance.

The distribution of pharmaceutical products is also subject to additional requirements and regulations, including extensive recordkeeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products. Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from U.S. government-funded healthcare programs, such as Medicare and Medicaid, or similar programs in other countries or jurisdictions, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the delay, reduction, termination or restructuring of our operations. Further, defending against any such actions can be costly and time-consuming, and may require significant financial and personnel resources. Therefore, even if we successfully defend against any such actions that may be brought against us, our business may be impaired. If any of the physicians or other providers or entities with whom we expect to do business is found to not comply with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs and imprisonment. If any of the above occur, it could adversely affect our ability to operate our business and our results of operations.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We participate in the Medicaid Drug Rebate Program, the 340B program, the U.S. Department of Veterans Affairs, Federal Supply Schedule, or FSS, pricing program, and the Tricare Retail Pharmacy program, which require us to disclose average manufacturer pricing, and, in the future may require us to report the average sales price for certain of our drugs to the Medicare program. Pricing

and rebate calculations vary across products and programs, are complex, and are often subject to interpretation by us, governmental or regulatory agencies and the courts. Furthermore, regulatory and legislative changes, and judicial rulings relating to these programs and policies (including coverage expansion), have increased and will continue to increase our costs and the complexity of compliance, have been and will continue to be time-consuming to implement, and could have a material adverse effect on our results of operations, particularly if CMS or another agency challenges the approach we take in our implementation. For example, in the case of our Medicaid pricing data, if we become aware that our reporting for a prior quarter was incorrect or has changed as a result of recalculation of the pricing data, we are generally obligated to resubmit the corrected data for up to three years after those data originally were due. Such restatements increase our costs and could result in an overage or underage in our rebate liability for past quarters. Price recalculations also may affect the ceiling price at which we are required to offer our products under the 340B program and give rise to an obligation to refund entities participating in the 340B program for overcharges during past quarters impacted by a price recalculation.

Civil monetary penalties can be applied if we are found to have knowingly submitted any false price or product information to the government, if we are found to have made a misrepresentation in the reporting of our average sales price, if we fail to submit the required price data on a timely basis, or if we are found to have charged 340B covered entities more than the statutorily mandated ceiling price. Additionally, our agreement to participate in the 340B program or our Medicaid drug rebate agreement could be terminated, in which case federal payments may not be available under Medicaid or Medicare Part D for our covered outpatient drugs. Additionally, if we overcharge the government in connection with our arrangements with FSS or Tricare Retail Pharmacy, we are required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges can result in allegations against us under the FCA and other laws and regulations. Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Further, legislation may be introduced that, if passed, would, among other things, further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting, and any additional future changes to the definition of average manufacturer price or the Medicaid rebate amount could affect our 340B ceiling price calculations and negatively impact our results of operations. Additionally, certain pharmaceutical manufacturers are involved in ongoing litigation regarding contract pharmacy arrangements under the 340B program. For example, on November 3, 2023, the U.S. District Court of South Carolina issued an opinion in Genesis Healthcare Inc. v. Becerra et al. that may lead to an expansion of the scope of patients eligible to access prescriptions at 340B pricing. The outcome of this and other judicial proceedings on the 340B program and the potential impact on the way in which manufacturers extend discounts to covered entities through contract pharmacies under the 340B program remain uncertain.

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other information processing worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Economic Area, or, EEA, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, and similarly, processing of personal data regarding individuals in the United Kingdom, or the UK, including personal health data, is subject to the UK General Data Protection Regulation and the UK Data Protection Act 2018, or, collectively the UK GDPR, and together with the EU GDPR, the GDPR. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining the consent of the individuals to whom the personal data relates, providing detailed information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EEA/UK that are not considered by the European Commission and the UK government as providing “adequate” protection to personal data, including the U.S., and, as a result, increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the U.S. Such transfers of personal data outside of the EEA and UK are prohibited unless a valid GDPR transfer mechanism (for example, the European Commission approved Standard Contractual Clauses, or SCCs, and the UK International Data Transfer Agreement/Addendum, or UK IDTA) has been put in place. Where relying on the SCCs /UK IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The international transfer obligations under the EEA/UK data protection regimes will require significant effort and cost, and may result in us needing to make strategic considerations around where EEA/UK personal data is transferred and which service providers we can utilize for the processing of EEA/UK personal data. Any inability to transfer personal data from the EEA and UK to the United States

in compliance with data protection laws may impede our ability to conduct trials and may adversely affect our business and financial position. The GDPR also permits data protection authorities to require the destruction of improperly gathered or used personal information and or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or €20 million (£17.5 million under the UK GDPR), whichever is greater and it also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Although the UK is regarded as a third country under the EU GDPR, the European Commission has now issued a decision recognizing the UK as providing adequate protection under the EU GDPR, or Adequacy Decision, and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted. The UK Government has introduced a Data Protection and Digital Information Bill, or UK Bill, into the UK legislative process to reform the UK's data protection regime, and if passed, the final version of the UK Bill may have the effect of further altering the similarities between the UK and EEA data protection regimes and threaten the UK Adequacy Decision from the European Commission, which may lead to additional compliance costs for us and could increase our overall risk. It is unclear how UK data protection laws and regulations will develop in the medium to longer term, and how data transfers to and from the UK will be regulated in the long term. Although the EU GDPR and the EU GDPR currently impose substantially similar obligations, it is possible that over the time the UK GDPR could become less aligned with the EU GDPR. In addition, EU member states have adopted national laws to supplement the EU GDPR, which may partially deviate from the EU GDPR, and the competent authorities in the EU Member States may interpret EU GDPR obligations slightly differently from country to country, such that we do not expect to operate in a uniform legal landscape in the EEA with respect to data protection regulations. The potential of the respective provisions and enforcement of the EU GDPR and UK GDPR further diverging in the future creates additional regulatory challenges and uncertainties for us. The lack of clarity on future UK laws and regulations and their interaction with EU laws and regulations could add legal risk, uncertainty, complexity and cost to the handling of European personal data and our privacy and data security compliance programs could require us to implement different compliance measures for the UK and EEA.

Similar legal requirements are either in place or are being proposed in the U.S. There are a broad variety of data protection laws that are applicable to our activities, and a wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General are all aggressive in reviewing consumers' privacy and data security protections. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act—which went into effect on January 1, 2020 and which was recently amended by the California Privacy Rights Act —is creating similar risks and obligations as those created by GDPR. Though the Act does exempt certain information collected as part of a clinical trial subject to the Federal Policy for the Protection of Human Subjects, or the Common Rule, it does apply to other personal information that we may otherwise handle, such as personal information collected in a business to business context and personal information collected from employees, applicants and retirees residing in California. Similar broad consumer privacy laws have already been passed in numerous states, and laws in Virginia, Colorado and Connecticut already have entered into force. In addition, bills for broad consumer privacy laws are being considered in numerous other states and at the federal level.

Compliance with the above requirements and any other data privacy and data security laws and regulations is a rigorous and time-intensive process and requires significant resources and an ongoing review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

Artificial intelligence presents risks and challenges that can impact our business including by posing security risks to our confidential information, proprietary information, and personal data.

Issues in the development and use of artificial intelligence, combined with an uncertain regulatory environment, may result in reputational harm, liability, or other adverse consequences to our business operations. As with many technological innovations, artificial intelligence presents risks and challenges that could impact our business. We may adopt and integrate generative artificial intelligence tools into our systems for specific use cases reviewed by legal and information security. Our vendors may incorporate generative artificial intelligence tools into their offerings without disclosing this use to us, and the providers of these generative artificial intelligence tools may not meet existing or rapidly evolving regulatory or industry standards with respect to privacy and data protection and may inhibit our or our vendors' ability to maintain an adequate level of service and experience. If we, our vendors, or our third-party partners experience an actual or perceived breach or privacy or security incident because of the use of generative artificial intelligence, we may lose valuable intellectual property and confidential information and our reputation and the public perception of the effectiveness of our security measures could be harmed. Further, bad actors around the world use increasingly sophisticated methods, including the use of artificial intelligence, to engage in illegal activities involving the theft and misuse of

personal information, confidential information, and intellectual property. Any of these outcomes could damage our reputation, result in the loss of valuable property and information, and adversely impact our business.

Risks Related to Our Intellectual Property

Our commercial success depends on our ability to protect our intellectual property and proprietary technology.

Our commercial success depends in large part on our ability to obtain and maintain intellectual property rights protection through patents, trademarks and trade secrets in the U.S. and other countries with respect to our proprietary product candidates, AMX0035, AMX0114, avexitide, and any future proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode, negate or preempt any competitive advantage we may have, which could harm our business and ability to sustain profitability. To protect our proprietary position, we have filed patent applications and may file other patent applications in the U.S. or abroad related to AMX0035, AMX0114, or any other current or future product candidates that are important to our business; we may also license or purchase patents or patent applications filed by others. With respect to protection of our intellectual property rights in avexitide, our acquisition of that product candidate from Eiger includes acquisition of all of Eiger's owned and co-owned patents and applications directed to avexitide, as well as assuming Eiger's licenses to patents and applications directed to avexitide and owned and co-owned by other entities. The patent application process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

If the scope of the patent protection we obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive advantage. We cannot provide any assurances that any of our patents have, or that any of our pending owned patent applications that mature into issued patents will include claims with a scope sufficient to protect our proprietary therapeutics or otherwise provide any competitive advantage. Other parties have developed or may develop technologies that may be related or competitive with our approach, and may have filed or may file patent applications and may have been issued or may be issued patents with claims that overlap or conflict with our patent applications, either by claiming the same compounds, formulations or methods or by claiming subject matter that could dominate our patent position. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the U.S. Furthermore, patents have a limited lifespan. In the U.S., the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to AMX0035, AMX0114, avexitide or any other current or future product candidates. In the event that an alternative combination of AMX0035, or TURSO as a single drug product, is developed and approved for use in indications for which we may seek approval and falls outside the scope of our patent claims, the marketability and commercial success of AMX0035 could be materially harmed.

Even if they are unchallenged, our owned patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapy that provides benefits similar to our product candidate but falls outside the scope of our patent protection or license rights. If the patent protection provided by the patent and patent applications we hold or pursue with respect to AMX0035, AMX0114, avexitide or any other current or future product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidate could be negatively affected, which would harm our business.

We, or any future partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position.

It is possible that defects of form in the preparation or filing of our patent or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we or our partners, collaborators, or licensees whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, or licensees are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

The patent position of biotechnology and pharmaceutical companies carries uncertainty. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which are dependent upon the current legal and intellectual property context, extant legal precedent and interpretations of the law by individuals. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are characterized by uncertainty.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the U.S., the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patent or pending patent applications, or that we were the first to file for patent protection of such inventions. If third parties have filed prior patent applications on inventions claimed in our patents or applications that were filed on or before March 15, 2013, an interference proceeding in the U.S. can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such prior applications after March 15, 2013, a derivation proceeding in the U.S. can be initiated by such third parties to determine whether our invention was derived from theirs.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents may be challenged in the courts or patent offices in the U.S. and abroad. Also, while we believe that we have disclosed all potentially relevant prior art relating to our patents and patent applications, there is no assurance that we have found all such prior art or disclosed it in every relevant jurisdiction. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all.

Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivation proceedings, ex parte reexaminations, inter partes review, supplemental examinations, or interference proceedings or challenges in district court, in the U.S. or in various foreign patent offices, including both national and regional, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of the patent or in patent or patent application claims being narrowed, invalidated or held unenforceable, in whole or in part, or in denial of the patent application or loss or reduction in the scope of one or more claims of the patent or patent application, any of which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products.

Pending and future patent applications may not result in patents being issued that protect our business, in whole or in part, or which effectively prevent others from commercializing competitive products. Competitors may also be able to design around our patents. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the U.S. For example, patent laws in various jurisdictions, including jurisdictions covering significant commercial markets, such as the European Patent Office, or EPO, China and Japan, restrict the patentability of methods of treatment of the human body more than U.S. law does. If these developments were to occur, they could have a material adverse effect on our ability to generate revenue.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our future development partners will be successful in protecting AMX0035, AMX0114, avexitide or any other current or future product candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance, whether intentional or not, can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;

- our competitors, many of whom have substantially greater resources and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, and sell AMX0035, AMX0114, or avexitide;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the U.S. may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates; and
- countries other than the U.S. may, under certain circumstances, force us to grant a license under our patents to a competitor, thus allowing the competitor to compete with us in that jurisdiction or forcing us to lower the price of our drug in that jurisdiction.

Issued patents that we have or may obtain or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors do not infringe our patents. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

In addition, we rely on the protection of our trade secrets and proprietary, unpatented know-how. Although we have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and invention assignment agreements with employees, consultants, collaborators, vendors, and advisors, we cannot provide any assurances that all such agreements have been duly executed, and third parties may still obtain this information or may come upon this or similar information independently. It is possible that technology relevant to our business will be independently developed by a person who is not a party to such a confidentiality or invention assignment agreement. We may not be able to prevent the unauthorized disclosure or use of our technical knowledge or trade secrets by consultants, collaborators, vendors, advisors, former employees and current employees. Furthermore, if the parties to our confidentiality agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a consequence of such breaches or violations. Our trade secrets could otherwise become known or be independently discovered by our competitors. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets. If any of these events occurs or if we otherwise lose protection for our trade secrets or proprietary know-how, our business may be harmed.

It is difficult and costly to protect our intellectual property and our proprietary technologies, and we may not be able to ensure their protection.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection for our proprietary product candidates, AMX0035, AMX0114, and our newly acquired product candidate, avexitide, as well as on successfully defending these patents against potential third-party challenges. Our ability to protect our product candidate from unauthorized making, using, selling, offering to sell or importing by third parties is dependent on the extent to which we have rights under valid and enforceable patents that cover these activities.

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved and have in recent years been the subject of much litigation. Changes in either the patent laws or in interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property. Over the past decade, U.S. federal courts have increasingly invalidated pharmaceutical and biotechnology patents during litigation often based on changing interpretations of patent law. Further, the determination that a patent application or patent claim meets all of the requirements for patentability is a subjective determination based on the application of law and jurisprudence. The ultimate determination by the U.S. Patent and Trademark Office, or USPTO, or by a court or other trier of fact in the U.S., or corresponding foreign national patent offices or courts, on whether a claim meets all requirements of patentability cannot be assured. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in our owned patents or patent applications.

We cannot provide assurances that any of our patent applications will be found to be patentable, including over our own prior art publications or patent literature, or will issue as patents. Neither can we make assurances as to the scope of any claims that may issue from our pending and future patent applications nor to the outcome of any proceedings by any potential third parties that could challenge the patentability, validity or enforceability of our patents and patent applications in the U.S. or foreign jurisdictions. Any such challenge, if successful, could limit patent protection for our products and current or future product candidates and/or materially harm our business.

In addition to challenges during litigation, third parties can challenge the validity of our patents in the U.S. using post-grant review and inter partes review proceedings, which some third parties have been using to cause the cancellation of selected or all claims of issued patents of competitors. For a patent filed March 16, 2013 or later, a petition for post-grant review can be filed by a third party in a nine-month window from issuance of the patent. A petition for inter partes review can be filed immediately following the issuance of a patent if the patent has an effective filing date prior to March 16, 2013. A petition for inter partes review can be filed after the nine-month period for filing a post-grant review petition has expired for a patent with an effective filing date of March 16, 2013 or later. Post-grant review proceedings can be brought on any ground of invalidity, whereas inter partes review proceedings can only raise an invalidity challenge based on published prior art and patents. These adversarial actions at the USPTO review patent claims without the presumption of validity afforded to U.S. patents in lawsuits in U.S. federal courts, and use a lower burden of proof than used in litigation in U.S. federal courts. Therefore, it is generally considered easier for a competitor or third party to have a U.S. patent invalidated in a USPTO post-grant review or inter partes review proceeding than invalidated in a litigation in a U.S. federal court. If any of our patents are challenged by a third party in such a USPTO proceeding, there is no guarantee that we will be successful in defending the patent, which may result in a loss of the challenged patent right to us.

In the EU, third parties can challenge the validity of our patents by filing an Opposition before the EPO. An adverse determination by the Opposition Board can result in the narrowing or invalidation of a European patent. If any of our European patents are challenged by a third party in such an opposition proceeding, there is no guarantee that we will be successful in defending the patent, which may result in a loss of the challenged patent right to us.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs;
- it is possible that one or more of our pending patent applications will not become an issued patent or, if issued, that the patent(s) claims will have sufficient scope to protect our technology, provide us with commercially viable patent protection or provide us with any competitive advantages;
- if our pending applications issue as patents, they may be challenged by third parties as invalid or unenforceable under U.S. or foreign laws;
- we may not successfully commercialize AMX0035, AMX0114, or avexitide before our relevant patents expire;
- we may not be the first to make the inventions covered by each of our patents and pending patent applications; or
- we may not develop additional proprietary technologies or product candidates that are separately patentable.

In addition, to the extent that we are unable to obtain and maintain patent protection for AMX0035, AMX0114, or avexitide or any other current or future product candidates or in the event that such patent protection expires, it may no longer be cost-effective to extend our portfolio by pursuing additional development of a product or product candidate for follow-on indications.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

In addition to patents, we also may rely on trade secrets to protect our proprietary product candidate, especially where we do not believe patent protection is appropriate or obtainable. Trade secrets are difficult to protect. We seek to protect our confidential proprietary information, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators. These agreements are designed to protect our proprietary information. However, we cannot be certain that such agreements have been entered into with all relevant parties, and we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. For example, our employees, consultants, contractors, outside scientific collaborators and other advisers may unintentionally or willfully disclose our information to competitors. Enforcing a claim that a third-party entity illegally obtained and is using any of our trade secrets is expensive and time-consuming, and the outcome is unpredictable, and we may not be able to obtain adequate remedies for such breaches. We also seek to

preserve the integrity and confidentiality of our confidential proprietary information by maintaining physical security of our premises and physical and electronic security of our IT systems, but it is possible that these security measures could be breached. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. Notably, proprietary technology protected by a trade secret does not preempt the patenting of independently developed equivalent technology, even if such equivalent technology is invented subsequent to the technology protected by a trade secret. If any of our confidential proprietary information were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Patent terms and market exclusivities, if obtained, may be inadequate to protect our competitive position on our products for an adequate amount of time.

Patents have a limited lifespan. In the U.S., if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. The patent term of a U.S. patent may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent.

Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

In the U.S., the Drug Price Competition and Patent Term Restoration Act of 1984 permits a Patent Term Extension, or PTE, of up to five years beyond the normal expiration of the patent to compensate patent owners for loss of enforceable patent term due to the lengthy regulatory approval process. PTE is limited to the approved indication (or any additional indications approved during the period of extension). We anticipate applying for PTE in the U.S. Similar extensions may be available in other countries where we are prosecuting patents and we likewise anticipate applying for such extensions.

The granting of such patent term extensions is not guaranteed and is subject to numerous requirements. We might not be granted an extension because of, for example, failure to apply within applicable periods, failure to apply prior to the expiration of relevant patents or failure to otherwise satisfy any of the numerous applicable requirements. Moreover, the applicable authorities, including the FDA and the USPTO in the U.S., and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to obtain approval of competing products following our patent expiration by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. If this were to occur, it could have a material adverse effect on our ability to generate revenue.

In addition, market exclusivities may be available for our product candidates and indications. If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval, subject to the requirement that the ANDA applicant certifies to the invalidity or non-infringement of any patents listed for our products in the Orange Book. If an infringement suit is timely filed by the NDA or patent holder, the FDA cannot finally approve the ANDA for 30 months unless a court decision in favor of the generic manufacturer is issued earlier. Three-year exclusivity is given to a drug if it contains an active moiety that has previously been approved, and the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are determined by the FDA to be essential to the approval of the NDA. This form of data exclusivity is known as New Clinical Investigation, or NCI, exclusivity. If AMX0035 is approved for future uses, such as Wolfram syndrome or PSP, and if avexitide is approved for future uses, or if current and future candidates, such as AMX0114, are approved with only NCI exclusivity, generic manufacturers may file their ANDAs anytime following approval of AMX0035, AMX0114, or avexitide and seek to launch their generic products following the expiration of the three year market exclusivity period, even if we still have patent protection for our product.

In addition, in the U.S. the FDCA provides a period of seven years of orphan drug exclusivity for drugs that treat small patient populations less than 200,000 patients or for which there are more than 200,000 patients but there is no reasonable expectation that the cost of developing and making the drug for such disease or condition will be recovered from sales in the U.S. of such drug.

In the EU, innovative medicinal products (including both small molecules and biological medicinal products), sometimes referred to as new active substances, or NAS, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents generic or biosimilar applicants from referencing the innovator's preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization, for a period of eight years from the date on which the reference product was first authorized in the EU. During the additional two-year period of market exclusivity, a generic or biosimilar marketing authorization can be submitted, and the innovator's

data may be referenced, but no generic or biosimilar product can be marketed until the expiration of the market exclusivity period. This 10-year market exclusivity period may be extended to 11 years if, during the first eight of those 10 years, the marketing authorization holder obtains an approval for one or more new therapeutic indications that bring significant clinical benefits compared with existing therapies. However, even if an innovative medicinal product gains the prescribed period of data exclusivity, another company may market another version of the product if such company obtained a marketing authorization based on an application with a complete and independent data package of pharmaceutical tests, preclinical tests and clinical trials. The current orphan medicines regime in the EU entitles an orphan medicine to a 10-year period of market exclusivity, which can be extended to 12 years if the sponsor complies with an agreed upon paediatric investigation plan. However, the European Commission introduced a legislative proposal in April 2023 that, if implemented, could reduce the current exclusivity period for certain orphan medicines.

Competition that AMX0035, AMX0114, avexitide or any future products, if approved, may face from generic versions of such products could negatively impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on our investments in those product candidates.

Changes in the interpretation of patent law in the U.S. and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

The U.S. Congress is responsible for passing laws establishing patentability standards. As with any laws, implementation is left to federal agencies and the federal courts based on their interpretations of the laws. Interpretation of patent standards can vary significantly within the U.S. Patent and Trademark Office, and across the various federal courts, including the Supreme Court. Recently, the Supreme Court has ruled on several patent cases, generally limiting the types of inventions that can be patented. Further, there are open questions regarding interpretation of patentability standards that the Supreme Court has yet to decisively address. Absent clear guidance from the Supreme Court, the USPTO has become increasingly conservative in its interpretation of patent laws and standards.

In addition to increasing uncertainty with regard to our ability to obtain patents in the future, the legal landscape in the U.S. has created uncertainty with respect to the value of patents. Depending on any actions by Congress, and future decisions by the lower federal courts and the Supreme Court, along with interpretations by the USPTO, the laws and regulations governing patents could change in unpredictable ways and could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not be able to enforce our intellectual property rights throughout the world.

Filing, prosecuting, enforcing and defending patents on our product candidate in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. The requirements for patentability may differ in certain countries, particularly in developing countries; thus, even in countries where we do pursue patent protection, there can be no assurance that any patents will issue with claims that cover our products.

Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the U.S. and the EU do not afford intellectual property protection to the same extent as the laws of the U.S. and the EU. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the U.S. and the EU or from selling or importing products made from our inventions in and into the U.S. or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop and market their own products and, further, may export otherwise infringing products to territories where we have patent protection if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Proceedings to enforce our patent rights, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Moreover, such proceedings could put our patents at risk of being invalidated or held unenforceable, or interpreted narrowly, and our pending patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products, if approved. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

Others may challenge inventorship or claim an ownership interest in our intellectual property which could expose it to litigation and have a significant adverse effect on its prospects.

A third party or former employee or collaborator may claim an inventorship or ownership interest in one or more of our or our licensors' patents or other proprietary or intellectual property rights. A third party could bring legal actions against us and seek monetary damages and/or enjoin clinical testing, manufacturing and marketing of the affected product or products. While we are presently unaware of any claims or assertions by third-parties with respect to inventorship or ownership of our patents or other intellectual property, we cannot guarantee that a third party will not assert a claim or an interest in any of such patents or intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to AMX0035, AMX0114, avexitide, or any other current or future product candidates. Further, regardless of the outcome, if we become involved in any litigation, it could consume a substantial portion of our resources, and cause a significant diversion of effort by our technical and management personnel.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing AMX0035, AMX0114, avexitide, or any other current or future product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidate without infringing the intellectual property and other proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Third parties may have U.S. and non-U.S. issued patents and pending patent applications relating to compounds, methods of manufacturing compounds and/or methods of use for the treatment of the disease indications for which we are developing AMX0035, AMX0114, avexitide, or any other current or future product candidates. If any third-party patents or patent applications are found to cover AMX0035, AMX0114, avexitide, or any other current or future product candidates or their methods of use or manufacture, we may not be free to manufacture or market such product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our products candidates, including patent infringement lawsuits in the U.S. or abroad. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the composition, use or manufacture of AMX0035, AMX0114, avexitide or any other current or future product candidates. While we perform periodic searches for relevant patents and patent applications with respect to our proprietary drug candidates, AMX0035, AMX0114, and our newly acquired product candidate, avexitide, we cannot guarantee that any of our patent searches or analyses including, but not limited to, the identification of relevant patents, the scope of patent claims or the expiration of relevant patents are complete or thorough, nor can we be certain that we have identified each and every patent and pending application in the U.S. and abroad that is relevant to or necessary for the commercialization of AMX0035, AMX0114, avexitide, or any other current or future product candidates in any jurisdiction. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that AMX0035, AMX0114, avexitide, or any other current or future product candidates may be accused of infringing. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Accordingly, third parties may assert infringement claims against us based on intellectual property rights that exist now or arise in the future. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use or manufacture. The scope of protection afforded by a patent is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidate, product or method either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the U.S., proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources, and we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate or product. If we were required to obtain a license to continue to manufacture or market the affected

product, we may be required to pay substantial royalties or grant cross-licenses to our patents. We cannot, however, assure you that any such license will be available on acceptable terms, if at all. Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations as a result of claims of patent infringement or violation of other intellectual property rights. Further, the outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance, including the demeanor and credibility of witnesses and the identity of any adverse party. This is especially true in intellectual property cases that may turn on the testimony of experts as to technical facts upon which experts may reasonably disagree. Furthermore, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively or additionally it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing AMX0035, AMX0114, avexitide, or any other current or future product candidates or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our current and former employees and our licensors' current and former employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including some which may be competitors or potential competitors. Some of these employees, including members of our senior management, may have executed proprietary rights, non-disclosure and non-competition agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may sustain damages or lose key personnel, valuable intellectual property rights or the personnel's work product, which could hamper or prevent commercialization of our technology, which in turn could materially affect our commercial development efforts. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while we typically require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our trademarks and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We rely on both registration and common law protection for our trademarks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. During trademark registration proceedings, we may receive rejections. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we propose to use for our products in the U.S. must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed product names, we may be required to expend significant additional resources in an effort to identify a usable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be

acceptable to the FDA. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Intellectual property rights do not necessarily address all potential threats to our business.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are competitive to AMX0035, for example a TURSO monotherapy, AMX0114, avexitide or any of our future product candidates but that are not covered by the claims of the patents that we own;
- others may independently develop similar or alternative technologies or otherwise circumvent any of our technologies without infringing our intellectual property rights;
- we or any of our collaborators might not have been the first to invent the inventions covered by the patents or patent applications that we own;
- we or any of our collaborators might not have been the first to file patent applications covering certain of the patents or patent applications that we or they own or have obtained a license, or will own or will have obtained a license;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- ownership of our patents or patent applications may be challenged by third parties;
- the patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business; and
- patent enforcement is expensive and time-consuming and difficult to predict; thus we may not be able to enforce any of our patents against a competitor.

Our reliance on third parties for research and development and manufacturing requires us to share our trade secrets, which increases the possibility that our trade secrets will be misappropriated or disclosed, and confidentiality agreements with employees and third parties may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We consider proprietary trade secrets or confidential know-how and unpatented know-how to be important to our business. We may rely on trade secrets or confidential know-how to protect our technology, especially where patent protection is believed by us to be of limited value. We rely on third parties for research and development work, and expect to rely on third parties for future manufacturing of our proprietary product candidate, AMX0035, AMX0114, and our newly acquired product candidate, avexitide, and any other current or future product candidates. We also expect to collaborate with third parties on the development of AMX0035, AMX0114, avexitide, and any other current or future product candidates. As a result of the aforementioned collaborations, we must, at times, share trade secrets with our collaborators.

Trade secrets or confidential know-how can be difficult to maintain as confidential. To protect this type of information against disclosure or appropriation by competitors, our policy is to require our employees, consultants, contractors and advisors to enter into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with us prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. However, current or former employees, consultants, contractors and advisers may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. The need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor’s discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have an adverse effect on our business and results of operations. Enforcing a claim that a third party obtained illegally and is using trade secrets or confidential know-how is expensive, time consuming and unpredictable. Moreover, the enforceability of confidentiality agreements may vary from jurisdiction to jurisdiction.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

We may need to acquire or license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development of additional future product candidates. It may be necessary for us to use the patented or proprietary technology of one or more third parties to commercialize our current and future product candidates. If we are unable to acquire such intellectual property outright, or obtain licenses to such intellectual property from such third parties when needed or on commercially reasonable terms, our ability to commercialize additional future product candidates, if approved, would likely be delayed.

The risks described elsewhere pertaining to our intellectual property rights also apply to the intellectual property rights that we may in-license, and any failure by us or our licensors to obtain, maintain, defend and enforce these rights could have an adverse effect on our business. In some cases we may not have control over the prosecution, maintenance or enforcement of the patents that we license, and may not have sufficient ability to provide input into the patent prosecution, maintenance and defense process with respect to such patents, and potential future licensors may fail to take the steps that we believe are necessary or desirable in order to obtain, maintain, defend and enforce the licensed patents.

Risks Related to Our Business Operations and Employee Matters

Our Restructuring Plan and associated organizational changes may not adequately reduce our operating costs or improve operating margins, may lead to additional workforce attrition, and may cause operational disruptions.

We implemented our Restructuring Plan that is designed to focus our resources on key clinical and preclinical programs in April 2024. The Restructuring Plan reduced our workforce by approximately 70% and a decreased external financial commitments outside of our priority areas.

The long-term effects of the Restructuring Plan may yield unintended consequences and costs, such as the loss of institutional knowledge and expertise, employee attrition beyond our intended reduction in force, a reduction in morale among our remaining employees, greater-than-anticipated costs incurred in connection with implementing the Restructuring Plan, and the risk that we may not achieve the benefits from the Restructuring Plan to the extent or as quickly as we anticipate, all of which may have a material adverse effect on our results of operations or financial condition. These restructuring initiatives could place substantial demands on our management and employees, which could lead to the diversion of our management's and employees' attention from other business priorities. In addition, while certain positions have been eliminated in connection with the Restructuring Plan, certain functions necessary to our reduced operations remain, and we may be unsuccessful in distributing the duties and obligations of departed employees among our remaining employees or to external service providers, which could result in disruptions to our operations. We may also discover that the workforce reduction and other restructuring efforts will make it difficult for us to pursue new opportunities and initiatives and require us to hire qualified replacement personnel, which may require us to incur additional and unanticipated costs and expenses. We may further discover that, despite the implementation of our Restructuring Plan, we may require additional capital to continue expanding our business, and we may be unable to obtain such capital on acceptable terms, if at all. Our failure to successfully accomplish any of the above activities and goals may have a material adverse impact on our business, financial condition, and results of operations. Finally, we have received and may receive additional lawsuits from employees relating to their separation from the Company. These lawsuits may result in increased costs and reputational risk to the Company.

We are continuously evaluating and pursuing strategic transactions, and may pursue strategic transactions in the future that are aligned with our mission in to improve our underlying business performance. For example, we have recently completed the acquisition of avexitide, and may in the future seek to acquire additional assets.

We anticipate completing acquisitions and business combinations in the future, especially given the discontinuation of the marketing authorizations for RELYVRIO/ALBRIOZA (AMX0035) for ALS and its withdrawal from the commercial market in the U.S. and Canada. For example, we recently completed the acquisition of substantially all of the rights, title and interests in, to and under those assets and interests used by Eiger in the development, manufacture and commercialization of avexitide. Our ability to complete future acquisitions and business combinations will depend, in part, on the availability of suitable candidates at acceptable prices, terms, and conditions; our ability to compete effectively for acquisition candidates; and the availability of capital and personnel to complete such acquisitions and run the acquired business effectively. Any acquisition or business combination could impair our business, reputation, operating results and financial condition. The benefits of an acquisition or business combination may take more time than expected to develop or integrate into our operations, and we cannot guarantee that previous or future acquisitions or business combinations will, in fact, produce any benefits. For example, we may not receive the anticipated benefits of the acquisition of avexitide for some time. Acquisitions and business combinations may involve a number of risks, the occurrence of which could adversely affect our business, reputation, operating results and financial condition, including:

- diversion of management's attention;
- disruption to our existing operations and plans;
- inability to effectively manage our expanded operations;
- difficulties or delays in integrating and assimilating information and financial systems, operations, manufacturing processes and products of an acquired business or other business venture or in realizing projected efficiencies, growth prospects, cost savings, and synergies;
- inability to successfully integrate or develop a distribution channel for acquired product lines;
- potential loss of key employees, customers, distributors, or sales representatives of the acquired businesses or adverse effects on existing business relationships with suppliers, customers, distributors, and sales representatives;
- adverse impact on overall profitability if our expanded operations do not achieve the financial results projected in our valuation models;

•assumption of contracts, liabilities and other agreements associated with acquired assets, including royalty or other payments due under such agreements;

•reallocation of amounts of capital from other operating initiatives and/or an increase in our leverage and debt service requirements to pay acquisition purchase prices or other business venture investment costs, which could in turn restrict our ability to access additional capital when needed or pursue other important elements of our business strategy;

•infringement by acquired businesses or other business ventures of intellectual property rights of others;

•violation of confidentiality, intellectual property and non-compete obligations or agreements by employees of an acquired business or lack of or inadequate formal intellectual property protection mechanisms in place at an acquired business;

•inaccurate assessment of additional post-acquisition investments, undisclosed, contingent or other liabilities or problems, unanticipated costs associated with an acquisition, and an inability to recover or manage such liabilities and costs;

•incorrect estimates made in the accounting for acquisitions and incurrence of non-recurring charges; and

•write-off of significant amounts of goodwill or other assets as a result of deterioration in the performance of an acquired business or product line, adverse market conditions, changes in the competitive landscape, changes in laws or regulations that restrict activities of an acquired business or product line, or as a result of a variety of other circumstances.

In addition, effective internal controls are necessary for us to provide reliable and accurate financial reports and to effectively prevent fraud. The integration of acquired businesses may result in our systems and controls becoming increasingly complex and more difficult to manage. We devote significant resources and time to comply with the internal control over financial reporting requirements of the Sarbanes-Oxley Act. However, we cannot be certain that these measures will ensure that we design, implement, and maintain adequate control over our financial processes and reporting in the future, especially in the context of acquisitions of other businesses, regardless of whether such acquired business was previously privately or publicly held. Any difficulties in the assimilation of acquired businesses into our control system could harm our operating results or cause us to fail to meet our financial reporting obligations. These risks, among others, could be heightened if we complete a large acquisition or other business combination or multiple transactions within a relatively short period of time.

We are currently operating in a period of economic uncertainty and capital markets disruption, which has been significantly impacted by geopolitical instability, ongoing military conflicts, including the ongoing war between Russia and Ukraine, the Israel-Hamas war and escalating conflict in the Middle East, U.S. presidential elections, events related thereto, such as changes to candidates or political unrest or otherwise, and high inflation and interest rates, any of which could have a material adverse effect on our business, financial condition and results of operations.

U.S. and global markets have recently been experiencing volatility and disruption caused by economic uncertainty, including as a result of the ongoing Russia-Ukraine war and the effects of sanctions imposed on Russia as a result of the conflict, as well as the Israel-Hamas war and escalating conflict in the Middle East. Although the length and impact of the ongoing military conflict is highly unpredictable, the conflict in Ukraine has led to market disruptions, including significant volatility in commodity prices, credit and capital markets, as well as supply chain interruptions, which contributed to record inflation globally. In addition, global markets may experience additional disruptions as a result of the current Israel-Hamas war and the escalating conflict in the Middle East. We are continuing to monitor inflation, the situations in Ukraine and Israel and global capital markets and assessing their potential impact on our business, including the impact on the supply chains we rely on for the manufacture of AMX0035, avexitide or any other current or future product candidates.

Although, to date, our business has not been materially impacted by the events described above, geopolitical tensions, or record inflation, it is impossible to predict the extent to which our operations will be impacted in the short and long term, or the ways in which such matters may impact our business. The extent and duration of the conflicts in Ukraine and Israel, geopolitical tensions, record inflation and resulting market disruptions are impossible to predict but could be substantial. Any such disruptions may also magnify the impact of other risks we face.

Inadequate funding for the FDA, the SEC and other government agencies, including from government shutdowns, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, the ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC

and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

We depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.

Our success depends, and will likely continue to depend, upon our ability to hire and retain the services of our current executive officers, principal consultants and others. We have entered into employment agreements with our current executive officers, but they may terminate their employment with us at any time. The loss of their services might impede the achievement of our research, development and commercialization objectives.

Our ability to compete in the biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. Our industry has experienced a high rate of turnover of management personnel in recent years, which has also impacted our company. For example, in February 2024, our then Chief Human Resource Officer, Debra Canner, was replaced by Linda Arsenault as our current Chief Human Resource Officer. Replacing executive officers or other key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize products successfully.

Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop AMX0035, avexitide or any other current or future product candidates will be limited.

We only have a limited number of employees to manage and operate our business.

We implemented a Restructuring Plan to reduce our workforce by 70% in April 2024. Our Restructuring Plan and our focus on research and the development of our product candidates requires us to optimize cash utilization and to manage and operate our business in a highly efficient manner. We cannot assure you that we will be able to hire and/or retain adequate staffing levels to develop our product candidates or to run our operations and/or to accomplish all of the objectives that we otherwise would seek to accomplish.

Our employees, independent contractors, consultants, collaborators and CROs may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators and CROs may engage in fraudulent conduct or other illegal activity. Misconduct by those parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates:

- FDA regulations or similar regulations of comparable non-U.S. regulatory authorities, including those laws requiring the reporting of true, complete and accurate information to such authorities;
- manufacturing standards;
- federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable non-U.S. regulatory authorities; and
- laws that require the reporting of financial information or data accurately.

Activities subject to these laws also involve the improper marketing, use or misrepresentation of information obtained in the course of clinical trials, creating fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of product materials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, disgorgement, integrity oversight and reporting obligations, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could have a material adverse effect on our ability to operate our business and our results of operations.

A pandemic, epidemic, or outbreak of an infectious disease may materially and adversely affect our business, including our preclinical studies, clinical trials, third parties on whom we rely, our supply chain, our ability to raise capital, our ability to conduct regular business and our financial results.

We are subject to risks related to public health crises. For instance, from 2020 through 2022, we experienced certain impacts from the COVID-19 pandemic, including alterations to our preclinical and clinical trial activities, such as scheduling certain work off-site and performing off-site assessments. There can be no guarantee we will not experience other impacts in the future, such as being forced to further delay or pause enrollment, experiencing potential interruptions to our supply chain, facing difficulties or additional costs in enrolling patients in future clinical trials or being able to achieve full enrollment of our studies within the timeframes we anticipate, or at all.

Any negative impact any future pandemic or similar disruption has on patient enrollment or treatment, or the development of AMX0035, avexitide and any other current or future product candidates, could cause costly delays to clinical trial activities, which could adversely affect our ability to obtain regulatory approval for and to commercialize AMX0035, avexitide and any other current or future product candidates, if approved, increase our operating expenses, which could have a material adverse effect on our financial results. The COVID-19 pandemic and other global macroeconomic factors have also caused significant volatility in public equity markets and disruptions to the U.S. and global economies and any future pandemic or similar disruption could lead to further market dislocation. Any such increased volatility and economic dislocation may make it more difficult for us to raise capital on favorable terms, or at all. If we or any of the third parties with whom we engage were to experience business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively affected, which could have a material adverse impact on our business and our results of operations and financial conditions. To the extent any future pandemic or similar disruption adversely affects our business and financial results, it may also heighten many of the other risks described in this ‘‘Risk Factors’’ section, such as those relating to the timing and completion of our clinical trials and our ability to obtain future financing.

Risks Related to Our Common Stock

The price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock may be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. For example, from January 7, 2022, the first day that our stock traded on the Nasdaq Global Select Market, through September 30, 2024, our stock has traded within a range of a high price of $41.93 and a low price of $1.58 per share. In addition to the factors discussed in this “Risk Factors” section and elsewhere in this Quarterly Report, these factors include:

- the commencement, enrollment or results of our ongoing and future preclinical studies and clinical trials, or any future preclinical studies or clinical trials, we may conduct of AMX0035, avexitide and any other current or future product candidates, or changes in the development status of our current and any future product candidates;
- any additional regulatory submissions for AMX0035, avexitide or any other current or future product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority’s review of such submissions, including without limitation the FDA’s issuance of a “refusal to file” letter or a request for additional information;
- adverse results or delays in our preclinical studies and clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial, or to terminate an existing clinical trial;

•adverse regulatory decisions, including failure to receive regulatory approvals for AMX0035, avexitide and any other current or future product candidates;

•withdrawal of products from the market;

•changes in laws or regulations applicable to current or future product candidates, including but not limited to clinical trial requirements for approvals;

•the failure to obtain coverage and adequate reimbursement of current or future product candidates, if approved;

•changes on the structure of healthcare payment systems;

•any changes to our relationship with any manufacturers, suppliers, licensors, future collaborators or other strategic partners;

•our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;

•our inability to establish collaborations, if needed;

•our failure to successfully commercialize AMX0035, avexitide and any other current or future product candidates;

•additions or departures of key scientific or management personnel;

•unanticipated serious safety concerns related to the use of AMX0035, avexitide and any other current or future product candidates;

•introduction of new products or services offered by us or our competitors, or the release or publication of clinical trial results from competing product candidates;

•announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us or our competitors;

•actual or anticipated variations in quarterly operating results;

•our cash position and rate of expenditures;

•our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;

•publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;

•changes in the market valuations of similar companies;

•overall performance of the equity markets;

•issuances of debt or equity securities;

•sales of our common stock by us or our stockholders in the future or the perception that such sales may occur;

•trading volume of our common stock;

•changes in accounting practices;

•ineffectiveness of our internal controls;

•disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;

•significant lawsuits, including patent or stockholder litigation;

•general political, geographical, and economic conditions, including the impact of global health crises such as the COVID-19 pandemic, historically high inflation, rising interest rates, the ongoing wars in Ukraine and Israel and U.S. presidential elections; and

•other events or factors, many of which are beyond our control.

In addition, the stock market in general, and pharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

Unstable market, economic, political and geographical conditions may have serious adverse consequences on our business, financial condition and stock price.

As widely reported, global credit and financial markets have experienced extreme volatility and disruptions in the past several years, including severely diminished liquidity and credit availability, declines in consumer confidence, increases in the rate of inflation and interest rates and uncertainty about economic stability, including most recently in connection with the wars in Ukraine and Israel and the U.S. presidential election. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. Our business could also be impacted by volatility caused by geopolitical events such as the wars in Ukraine and Israel. If the current equity and credit markets deteriorate, or do not improve, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay, scale back or discontinue the development and commercialization of one or more of our product candidates or delay our pursuit of potential in-licenses or acquisitions. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget.

We are no longer an emerging growth company and the reduced compliance requirements applicable to emerging growth companies no longer apply to us.

We no longer qualify as an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and as such we no longer are entitled to rely on exemptions from certain compliance requirements that are applicable to companies that are emerging growth companies. As a result, subject to certain grace periods, we are now required to:

- engage an independent registered public accounting firm to provide an attestation report on our internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act of 2002;
- submit certain executive compensation matters to stockholder advisory votes; and
- disclose a compensation discussion and analysis, including disclosure regarding certain executive compensation related items such as the correlation between executive compensation and performance and comparisons of the chief executive officer's compensation to median employee compensation.

We are no longer able to take advantage of cost savings associated with the JOBS Act. Furthermore, if the additional requirements applicable to non-emerging growth companies divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss and may require us to reduce costs in other areas of our business. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. Furthermore, if we are unable to satisfy our obligations as a non-emerging growth company, we could be subject to delisting of our common stock, fines, sanctions and other regulatory action and potentially civil litigation.

Commencing December 31, 2024, we will be a smaller reporting company due to our requalification at June 30, 2024 and we will become an accelerated filer, rather than a large accelerated filer due to our status at June 30, 2024. We cannot be certain if the reduced reporting requirements applicable to smaller reporting companies will make our common stock less attractive to investors.

As of June 30, 2023, the market value of our common stock that was held by non-affiliates exceeded $700 million, and therefore, effective as of January 1, 2024, we became a large accelerated filer. We also were no longer qualified as an emerging growth company or smaller reporting company and were no longer able to avail ourselves of the reduced disclosure requirements available to smaller reporting companies. However, based on the market value of our common stock that was held by non-affiliates as of June 30, 2024, we will regain smaller reporting company status effective as of December 31, 2024 and will be able to avail ourselves of the reduced disclosure requirements. As a smaller reporting company, we will be permitted and intend to rely on reduced disclosure requirements that are applicable to other public companies that are smaller reporting companies. Smaller reporting companies are able to provide simplified executive compensation disclosure and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide supplemental financial information or risk factors. Despite status effectiveness at December 31, 2024, due to requalification we are able to rely on these reduced requirements beginning after June 30, 2024. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active

trading market for our common stock and our stock price may be more volatile. As of June 30, 2024, we are also an accelerated filer and are still required to have our independent auditors annually attest to our evaluation, as well as issue their own opinion on our internal control over financial reporting.

A significant portion of our total outstanding shares may be sold into the market, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of October 31, 2024, we had outstanding 68,547,860 shares of common stock, which may be resold in the public market immediately without restriction, unless held by our affiliates. Moreover, holders of approximately 11.9 million shares of our common stock have rights, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our recent or any future earnings, if any, to finance the operation, development and growth of our business. In addition, the terms of any future debt or credit agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding common stock, own a significant portion of our common stock. As a result, these stockholders acting together, could be able to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, could significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that our stockholders may feel are in their best interest.

Delaware law and provisions in our amended and restated certificate of incorporation, or our certificate of incorporation, and amended and restated bylaws, or our bylaws, could make a merger, tender offer or proxy contest difficult, thereby depressing the trading price of our common stock.

Provisions of our certificate of incorporation and bylaws may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our common stock. Among other things, our certificate of incorporation and bylaws:

- permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate (including the right to approve an acquisition or other change in our control);
- provide that the authorized number of directors may be changed only by resolution of the board of directors;
- provide that our board of directors or any individual director may only be removed with cause and the affirmative vote of the holders of at least 66-2/3% of the voting power of all of our then-outstanding common stock;
- provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- divide our board of directors into three classes;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner and also specify requirements as to the form and content of a stockholder's notice;

•do not provide for cumulative voting rights (therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose);

•provide that special meetings of our stockholders may be called only by the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors; and

•provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for the following types of actions or proceedings under state, statutory and common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (iii) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws; (iv) any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws; (v) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers or other employees governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants; provided these provisions of our certificate of incorporation and bylaws will not apply to suits brought to enforce a duty or liability created by the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction; and provided that, unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the U.S. shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act of 1933, as amended, or the Securities Act.

The amendment of any of these provisions, with the exception of the ability of our board of directors to issue shares of preferred stock and designate any rights, preferences and privileges thereto, would require approval by the holders of at least 66-2/3% of our then-outstanding common stock.

In addition, as a Delaware corporation, we are subject to Section 203 of the Delaware General Corporation Law. These provisions may prohibit large stockholders, in particular those owning 15% or more of our outstanding voting stock, from merging or combining with us for a certain period of time. A Delaware corporation may opt out of this provision by express provision in its original certificate of incorporation or by amendment to its certificate of incorporation or bylaws approved by its stockholders. However, we have not opted out of this provision.

These and other provisions in our certificate of incorporation, bylaws and Delaware law could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by our then-current board of directors, including delay or impede a merger, tender offer or proxy contest involving our company. The existence of these provisions could negatively affect the price of our common stock and limit opportunities for you to realize value in a corporate transaction.

If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, investors' views of us and, as a result, the value of our common stock.

Pursuant to Section 404, our management is required to assess and report annually on the effectiveness of our internal control over financial reporting and to identify any material weaknesses in our internal control over financial reporting. As a result of no longer qualifying as an emerging growth company as defined in the JOBS Act and becoming a large accelerated filer, we were also required to comply with, among other requirements, the auditor attestation requirements of Section 404(b), and as an accelerated filer will be required to continue to comply with such requirements. Preparing such attestation report and the cost of compliance with reporting requirements that we had not previously implemented has and will continue to increase our expenses and require significant management time. Investors may find our common stock less attractive because of the additional compliance costs. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

The rules governing the standards that must be met for management and our independent registered public accounting firm to assess our internal control over financial reporting are complex and require significant documentation, testing, and possible remediation. In connection with our and our independent registered public accounting firm's evaluations of our internal control over financial reporting, we may need to upgrade systems, including information technology, implement additional financial and management controls, reporting systems, and procedures, and hire additional accounting and finance staff.

Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. In addition, any testing by us or our independent registered public accounting firm conducted in connection with Section 404 may reveal deficiencies in our internal control over financial reporting that are deemed to be material

weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock. Internal control deficiencies could also result in a restatement of our financial results in the future. We could become subject to stockholder or other third-party litigation, as well as investigations by the SEC, the Nasdaq Global Select Market, or other regulatory authorities, which could require additional financial and management resources and could result in fines, trading suspensions, payment of damages or other remedies. Further, any delay in compliance with the auditor attestation provisions of Section 404 could subject us to a variety of administrative sanctions, including ineligibility for short-form resale registration, action by the SEC and the suspension or delisting of our common stock, which could reduce the trading price of our common stock and could harm our business.

Our bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the U.S. will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our bylaws provide that, to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants, the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action or proceeding asserting a breach of fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders;
- any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees arising out of or pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or bylaws;
- any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws;
- any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and
- any action asserting a claim against us or any of our directors, officers or other employees that is governed by the internal affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our certificate of incorporation further provides that the federal district courts of the U.S. will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive forum provision in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that we will need significant additional capital in the future to continue our planned operations, including conducting clinical trials, commercialization efforts if we are able to obtain marketing approval of any of AMX0035, avexitide or any other current or future product candidates, research and development activities, and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors

could gain rights, preferences and privileges senior to the holders of our common stock, including shares of common stock sold in our public offerings.

Pursuant to our 2022 Stock Option and Incentive Plan, or the 2022 Plan, our management is authorized to grant stock options to our employees, directors and consultants. Additionally, the number of shares of our common stock reserved for issuance under our 2022 Plan will automatically increase on January 1 of each year, beginning on January 1, 2023 and continuing through and including January 1, 2032, by 5.0% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. In addition, pursuant to our ESPP, the number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, beginning on January 1, 2023 (through January 1, 2032), by the lesser of (i) 1.0% of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of the automatic increase, and (ii) 1,210,000 shares; provided that before the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

General Risk Factors

We incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives.

As a public company, we incur significant and ongoing legal, accounting, and other expenses, particularly now that we are no longer an emerging growth company. We are subject to the reporting requirements of the Exchange Act, which require, among other things, that we file with the SEC annual, quarterly, and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and the Nasdaq Global Select Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

Moreover, since we ceased to be an emerging growth company, we may no longer take advantage of certain exemptions from various reporting requirements that are applicable to public companies. This increase in reporting requirements will further increase our compliance burden. We expect to continue to incur substantial costs to comply with the rules and regulations applicable to public companies. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition, and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

Cyber-attacks or other failures in our telecommunications or IT systems, or those of our collaborators, CROs, third-party logistics providers, distributors or other contractors or consultants, could result in information theft, data corruption and significant disruption of our business operations.

We, our collaborators, our CROs, third-party logistics providers, distributors and other contractors and consultants utilize IT systems and networks to process, transmit and store electronic information in connection with our business activities. As use of digital technologies has increased, cyber incidents, including third parties gaining access to employee accounts using stolen or inferred credentials, computer malware, viruses, social engineering, spamming or other means, and deliberate attacks and attempts to gain unauthorized access to computer systems and networks, have generally been increasing in frequency and sophistication. Cyber-attacks also could include phishing attempts or e-mail fraud to, for example, cause payments or information to be transmitted to an unintended recipient. We and some of our third-party collaborators have in the past and may in the future experience cyber security attacks. These threats pose a risk to the security of our, our collaborators’, our CROs’, third-party logistics providers’, distributors’ and other contractors’ and consultants’ systems and networks, and the confidentiality, availability and integrity of our data. There can be no assurance that we will be successful in preventing cyber-attacks or successfully mitigating their effects. Similarly, there can be no assurance that our collaborators, CROs, third-party logistics providers, distributors and other contractors and consultants will be successful in protecting our clinical and other data that is stored on their systems or to which they have access. Any cyber-attack, data breach, security incident or destruction, misuse, or loss of data could result in a violation of applicable U.S. and international privacy, data protection and other laws, and subject us to litigation and governmental investigations and proceedings by federal, state and local regulatory entities in the U.S. and by international regulatory entities, resulting in exposure to material civil and/or criminal liability. Further, our general liability insurance and corporate risk program may not cover all potential claims to which we are exposed and may not be adequate to indemnify us for all liability that maybe imposed and could have a material adverse effect on our business and prospects. For example, the loss of clinical trial data from completed, ongoing or future clinical trials for AMX0035, avexitide or any of our future product candidates could result in delays in our development and regulatory approval efforts and significantly increase our costs to recover or reproduce the data. In addition, we may suffer reputational harm or face litigation or adverse regulatory action as a result of cyber-attacks or other data security breaches or incidents and may incur reputational harm and significant additional expense, including to implement further data protection or remedial measures, from fines and penalties or other liability, and from loss of existing and future business.

Our ability to use net operating losses and research and development credits to offset future taxable income may be subject to certain limitations.

As of December 31, 2023, we had U.S. federal net operating loss, or NOL, carryforwards of $69.8 million that carry forward indefinitely. The amount of annual utilization of these NOL carryforwards may be limited based on provisions of the Tax Cuts and Jobs Act of 2017, or TCJA. As of December 31, 2023, we also had U.S. federal research and development tax credit carryforwards of $6.8 million and we have additionally recorded deferred tax assets for U.S. state NOL and research and development tax credit carryforwards of $9.8 million. These U.S. federal research and development tax credit and U.S. state carryforwards could begin to expire if unused in 2042 and 2035, respectively. Utilization of all NOL and research and development tax credit carryforwards is conditioned upon us generating U.S. federal and state taxable income.

Ownership changes occurred in the years ended December 31, 2016 and 2023. In general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the IRC, and corresponding provisions of state law, a corporation that undergoes an ownership change is subject to limitations on its ability to utilize its pre-change NOL or tax credit carryforwards to offset future taxable income. For these purposes, an ownership change generally occurs where the aggregate stock ownership of one or more stockholders or groups of stockholders who owns at least five percent of a corporation's stock increases its ownership by more than 50 percentage points over its lowest ownership percentage within a specified testing period. Future changes in our stock ownership, many of which are outside of our control, could result in an ownership change under Sections 382 and 383 of the IRC. Our existing federal and state NOL and research and development tax credit carryforwards may be subject to limitations arising from these future ownership changes. Accordingly, we may not be able to utilize a material portion of these carryforwards.

We are currently involved in securities class action litigation and could be subject to additional securities class action litigation in the future.

On February 9, 2024, a putative class action lawsuit was filed in the U.S. District Court for the Southern District of New York against the Company and certain of its current and former officers (*Shih v. Amylyx Pharmaceuticals, Inc., et al.*, Case Number 1:24-CV-00988, or the Shih Complaint). Plaintiff filed an amended complaint on June 24, 2024. The Shih Complaint asserts a claim against all defendants for alleged violations of Section 10(b) of the Exchange Act and Rule 10b-5 promulgated thereunder and a claim under Section 20(a) against certain current and former officers as alleged controlling persons. The Shih Complaint alleges that defendants made materially false and misleading statements related to the commercial results and prospects for RELYVRIO. The Shih Complaint seeks unspecified damages, interest, costs and attorneys' fees, and other unspecified relief that the court deems appropriate.

On August 12, 2024, the case was transferred from the U.S. District Court for the Southern District of New York to the U.S. District Court for the District of Massachusetts, or the Court, and assigned docket number 1:24-CV-12068. Following the transfer, on September 6, 2024, defendants moved to dismiss the Shih Complaint. Plaintiff filed his opposition to Defendants' motion to dismiss on October 21, 2024 and defendants' response is due on or before November 20, 2024. On October 2, 2024, a derivative complaint was filed in the U.S. District Court for the District of Massachusetts against certain current and former director and officer defendants, naming the Company as nominal defendant (*Jones v. Cohen, et al.*, 1:24-CV-12527, or the Derivative Complaint). The substantive allegations mirror those of the Shih Complaint but also include claims for alleged violations of Section 14(a) of the Exchange Act, breach of fiduciary duty, insider trading, and unjust enrichment. The Derivative Complaint seeks unspecified damages to be awarded to the Company along with interest, costs, and attorneys' fees, restitution, and unspecified corporate governance and internal procedural reforms and improvements. On October 31, 2024, the Court entered an order staying the action until the earlier of the dismissal of the Shih Complaint with prejudice, including the exhaustion of all appeals, or defendants file an answer to the Shih Complaint.

The Company intends to defend against the Shih Complaint and Derivative Complaint vigorously. At this time, an estimate of the impact, if any, of the claims made in the Shih Complaint and Derivative Complaint cannot be made.

We may also become subject to additional securities class action lawsuits in the future. Securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. Such litigation could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

Our failure to meet Nasdaq's continued listing requirements could result in a delisting of our common stock.

If we fail to satisfy the continued listing requirements of the Nasdaq Global Select Market, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase our common stock when you wish to do so. In the event of a delisting, we can provide no assurance that any action taken by us to restore

compliance with listing requirements would allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping below the minimum bid price requirement or prevent future non-compliance with the listing requirements of the Nasdaq Global Select Market.

If securities analysts publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock depends in part on the research and reports that industry or securities analysts publish about us or our business. If one or more of the analysts who may cover us issues an adverse opinion about our company, our stock price would likely decline. If one or more of these analysts ceases research coverage of us or fails to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

Item 2. Unregistered Sales of Equity Securities, Use of Proceeds and Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

(c)

During the three months ended September 30, 2024, no officers or directors of the Company (as defined in Rule 16a-1(f)) adopted or terminated a “Rule 10b5-1 trading arrangement” or a “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K of the Exchange Act, except as described below:

- James Frates, our Chief Financial Officer, terminated a Rule 10b5-1 trading plan on September 10, 2024, which was scheduled to expire on December 1, 2024. The Rule 10b5-1 trading plan, which was adopted on December 14, 2023 to satisfy the affirmative defense conditions of Rule 10b5-1(c), provided for the sale of up to 90,000 shares of our common stock.
- George Milne, a member of our board of directors, terminated a Rule 10b5-1 trading plan on September 16, 2024, which was scheduled to expire on December 1, 2024. The Rule 10b5-1 trading plan, which was adopted on September 15, 2023 to satisfy the affirmative defense conditions of Rule 10b5-1(c), provided for the sale of up to 106,000 shares of our common stock.
- Gina M. Mazzariello, our Chief Legal Officer and General Counsel, terminated a Rule 10b5-1 trading plan on September 19, 2024, which was scheduled to expire on March 8, 2025. The Rule 10b5-1 trading plan, which was adopted on December 14, 2023 to satisfy the affirmative defense conditions of Rule 10b5-1(c), provided for the sale of up to 76,290 shares of our common stock.

Item 6. Exhibits.

Exhibit Number	Description
3.1	Fourth Amended and Restated Certificate of Incorporation of Amylyx Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on January 11, 2022).
3.2	Second Amended and Restated Bylaws of Amylyx Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on January 11, 2022).
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.3*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1+	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2+	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.3+	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith.
+ This certification will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, except to the extent specifically incorporated by reference into such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

AMYLYX PHARMACEUTICALS, INC.

Date: November 7, 2024

By: /s/ Joshua B. Cohen
Joshua B. Cohen
Co-Chief Executive Officer

By: /s/ Justin Klee
Justin Klee
Co-Chief Executive Officer

By: /s/ James M. Frates
James M. Frates
Chief Financial Officer

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Joshua B. Cohen, certify that:

1.I have reviewed this Quarterly Report on Form 10-Q of Amylyx Pharmaceuticals, Inc.;

2.Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3.Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4.The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

i.Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

ii.Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

iii.Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

iv.Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5.The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

i.All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

ii.Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 7, 2024

By: /s/ **Joshua B. Cohen**
Joshua B. Cohen
Co-Chief Executive Officer

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Justin B. Klee, certify that:

1.I have reviewed this Quarterly Report on Form 10-Q of Amylyx Pharmaceuticals, Inc.;

2.Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3.Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4.The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

i.Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

ii.Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

iii.Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

iv.Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5.The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

i.All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

ii.Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 7, 2024

By: /s/ **Justin B. Klee**
Justin B. Klee
Co-Chief Executive Officer

Exhibit 31.3

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, James M. Frates, certify that:

1.I have reviewed this Quarterly Report on Form 10-Q of Amylyx Pharmaceuticals, Inc.;

2.Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3.Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4.The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

i.Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

ii.Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

iii.Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

iv.Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5.The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

i.All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

ii.Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 7, 2024

By: /s/ **James M. Frates**
James M. Frates
Chief Financial Officer

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Amylyx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending September 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1)The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2)The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: November 7, 2024

By: /s/ **Joshua B. Cohen**
Joshua B. Cohen
Co-Chief Executive Officer

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Amylyx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending September 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1)The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2)The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: November 7, 2024

By: /s/ **Justin B. Klee**
Justin B. Klee
Co-Chief Executive Officer

Exhibit 32.3

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Amylyx Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending September 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

(1)The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2)The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: November 7, 2024

By: /s/ **James M. Frates**
James M. Frates
Chief Financial Officer