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 June 30, 2022

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)**

Registrant's telephone number, including area code: (617) 682-0917

N/A

(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 August 8, 2022, the registrant had 58,533,226 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 JUNE 30, 2022

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q ("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 ("the Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended ("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,” “project,” “should,” “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 and maintain regulatory approval of AMX0035 (also known as ALBRIOZA in Canada) and any future product candidates;
- our ability to successfully commercialize and market AMX0035 and any future 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 commercial supplies;
- the potential market size, opportunity and growth potential for AMX0035 and any future product candidates, if approved;
- our ability to build our own sales and marketing capabilities, or seek collaborative partners, to commercialize AMX0035 and any future product candidates, if approved;
- our ability to obtain funding for our operations;
- the initiation, timing, progress and results of our preclinical studies and clinical trials, including our Phase 3 clinical trial of AMX0035 for the treatment of ALS, known as the PHOENIX trial, and our research and development activities;
- our ability to retain the continued service of our key professionals and to identify, hire and retain additional qualified professionals;
- our ability to advance AMX0035 and any future product candidates into, and successfully complete, 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 pricing and reimbursement of AMX0035 in Canada and in any other jurisdictions in which AMX0035 is approved, if any, and of any other product candidates, if approved;
- the rate and degree of market acceptance AMX0035 and any future product candidates by physicians, patients, third-party payors and others in the medical community;
- the implementation of our business model, strategic plans for our business, product candidates and technology;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- developments relating to our competitors and our industry, including any regulatory developments;
- our estimates regarding expenses, capital requirements, cash runway and future and needs for additional financing;
- our financial performance; and
- other statements about future events, including those made in our Annual Report on Form 10-K for the fiscal year ended December 31, 2021 ("Annual Report").

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 Part II, Item 1A, “Risk Factors” and elsewhere in this Quarterly Report, as well as in Part I, Item 1A. and elsewhere in our Annual 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 ("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)

	June 30, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 67,440	$ 50,191
Short-term investments	139,241	45,927
Prepaid expenses and other current assets	9,458	5,392
Deferred offering costs	—	3,441
Total current assets	216,139	104,951
Property and equipment, net	1,857	474
Restricted cash	419	189
Operating lease right-of-use assets	6,369	—
Other assets	456	—
Total assets	$ 225,240	$ 105,614
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 8,117	$ 4,372
Accrued expenses and other current liabilities	17,260	13,024
Operating lease liabilities, current portion	1,787	—
Total current liabilities	27,164	17,396
Operating lease liabilities, net of current portion	5,287	—
Deferred rent	—	35
Total liabilities	32,451	17,431
Commitments and contingencies (Note 13)		
Series A redeemable convertible preferred stock, $0.0001 par value; 0 and 6,289,609 shares authorized, issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	—	7,675
Series B redeemable convertible preferred stock, $0.0001 par value; 0 and 15,100,000 shares authorized as of June 30, 2022 and December 31, 2021, respectively; 0 and 14,496,835 shares issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	—	64,387
Series C-1 redeemable convertible preferred stock, $0.0001 par value; 0 and 13,150,430 shares authorized, issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	—	134,791
Series C-2 redeemable convertible preferred stock, $0.0001 par value; 0 and 3,170,585 shares authorized, issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	—	32,498
Stockholders' equity (deficit):		
Preferred stock, $0.0001 par value; 10,000,000 and 0 shares authorized as of June 30, 2022 and December 31, 2021, respectively; 0 shares issued or outstanding as of June 30, 2022 and December 31, 2021	—	—
Common stock, $0.0001 par value; 300,000,000 and 56,500,000 shares authorized as of June 30, 2022 and December 31, 2021, respectively; 58,533,226 and 7,020,487 shares issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	6	1
Additional paid-in capital	450,739	4,667
Accumulated deficit	(257,760)	(155,845)
Accumulated other comprehensive (loss) income	(196)	9
Total stockholders' equity (deficit)	192,789	(151,168)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 225,240	$ 105,614

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 June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 24,259	$ 10,929	$ 45,723	$ 17,793
General and administrative	29,994	7,658	56,344	13,662
Total operating expenses	54,253	18,587	102,067	31,455
Loss from operations	(54,253)	(18,587)	(102,067)	(31,455)
Other income (expense), net:				
Interest income	402	1	533	3
Change in fair value of convertible notes	—	(3,310)	—	(5,228)
Other (expense) income, net	(42)	(26)	(61)	235
Total other income (expense), net	360	(3,335)	472	(4,990)
Loss before income taxes	(53,893)	(21,922)	(101,595)	(36,445)
Provision for income taxes	174	—	320	—
Net loss	$ (54,067)	$ (21,922)	$ (101,915)	$ (36,445)
Net loss per share attributable to common stockholders —basic and diluted	$ (0.93)	$ (3.41)	$ (1.85)	$ (5.75)
Weighted-average shares used in computing net loss per share attributable to common stockholders—basic and diluted	58,275,903	6,433,889	54,958,537	6,334,813

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Net loss	$ (54,067)	$ (21,922)	$ (101,915)	$ (36,445)
Other comprehensive loss:				
Foreign currency translation adjustment	58	—	(10)	—
Net unrealized loss on investments held	(103)	—	(195)	—
Other comprehensive loss	(45)	—	(205)	—
Comprehensive loss	$ (54,112)	$ (21,922)	$ (102,120)	$ (36,445)

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

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)
(in thousands, except share data)
(Unaudited)

	Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Series C-1 Redeemable Convertible Preferred Stock		Series C-2 Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Capital	Loss	Deficit	Deficit
Balance as of December 31, 2021	6,289,609	$ 7,675	14,496,835	$ 64,387	13,150,430	$ 134,791	3,170,585	$ 32,498	7,020,487	$ 1	$ 4,667	$ 9	$ (155,845)	$ (151,168)
Conversion of preferred stock into common stock upon initial public offering	(6,289,609)	(7,675)	(14,496,835)	(64,387)	(13,150,430)	(134,791)	(3,170,585)	(32,498)	39,474,330	4	239,347	—	—	239,351
Issuance of common stock upon initial public offering, net of issuance costs of $19,639	—	—	—	—	—	—	—	—	11,369,369	1	196,378	—	—	196,379
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	4,392	—	—	4,392
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(160)	—	(160)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(47,848)	(47,848)
Balance as of March 31, 2022	—	$ —	—	$ —	—	$ —	—	$ —	57,864,186	$ 6	$ 444,784	$ (151)	$ (203,693)	$ 240,946
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	669,040	—	248	—	—	248
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	5,707	—	—	5,707
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	(45)	—	(45)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(54,067)	(54,067)
Balance as of June 30, 2022	—	$ —	—	$ —	—	$ —	—	$ —	58,533,226	$ 6	$ 450,739	$ (196)	$ (257,760)	$ 192,789

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

AMYLYX PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)
(in thousands, except share data)
(Unaudited)

	Series A Redeemable Convertible Preferred Stock		Series B Redeemable Convertible Preferred Stock		Series C-1 Redeemable Convertible Preferred Stock		Series C-2 Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Capital	Loss	Deficit	Deficit
Balance as of December 31, 2020	6,289,609	$ 7,675	14,496,835	$ 64,387	—	$ -	—	$ -	6,137,206	$ 1	$ 1,188	$ -	$ (67,914)	$ (66,725)
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	290,032	—	31	—	—	31
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	586	—	—	586
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(14,523)	(14,523)
Balance as of March 31, 2021	6,289,609	$ 7,675	14,496,835	$ 64,387	—	$ —	—	$ —	6,427,238	$ 1	$ 1,805	$ —	$ (82,437)	$ (80,631)
Issuance of common stock upon exercise of stock options	—	—	—	—	—	—	—	—	9,684	—	3	—	—	3
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	657	—	—	657
Other comprehensive loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(21,922)	(21,922)
Balance as of June 30, 2021	6,289,609	$ 7,675	14,496,835	$ 64,387	—	$ —	—	$ —	6,436,922	$ 1	$ 2,465	$ —	$ (104,359)	$ (101,893)

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)

	Six Months Ended June 30,	
	2022	2021
Cash flows used in operating activities:		
Net loss	$ (101,915)	$ (36,445)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	10,099	1,243
Depreciation expense	140	20
Net amortization of premiums and discounts on investments	(107)	—
Change in fair value of convertible notes	—	5,228
Changes in operating assets and liabilities:		
Interest receivable	(13)	(2)
Prepaid expenses and other current assets	(4,053)	(665)
Operating lease right-of-use assets	790	—
Other assets	(456)	125
Accounts payable	3,755	(872)
Accrued expenses and deferred rent	4,784	5,678
Operating lease liabilities	(120)	—
Net cash used in operating activities	(87,096)	(25,690)
Cash flows used in investing activities:		
Purchases of property and equipment	(1,447)	(14)
Purchases of investments	(154,313)	—
Proceeds from maturities of short-term investments	60,911	—
Net cash used in investing activities	(94,849)	(14)
Cash flows provided by financing activities:		
Forgiveness of PPP loan	—	(263)
Proceeds from initial public offering	200,897	—
Initial public offering costs paid	(1,518)	(80)
Proceeds from issuance of convertible notes—related parties	—	14,272
Proceeds from issuance of convertible notes	—	11,887
Proceeds from exercise of stock options	248	34
Proceeds received in advance of issuance of Series C preferred stock	—	2,070
Net cash provided by financing activities	199,627	27,920
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(203)	2
Net increase in cash, cash equivalents and restricted cash	17,479	2,218
Cash, cash equivalents and restricted cash, beginning of period	50,380	13,066
Cash, cash equivalents and restricted cash, end of period	$ 67,859	$ 15,284
Supplemental disclosure of cash flow information:		
Unrealized loss on short-term investments	$ 195	$ —
Purchases of property and equipment included in accounts payable	$ 76	$ 29
Right-of-use assets and liabilities upon ASC842 adoption	$ 2,201	$ —
Right-of-use assets obtained in exchange for lease liabilities	$ 4,958	$ —
Movement of deferred offering costs to equity	$ 4,518	$ —
Initial public offering costs included in accounts payable and accrued expenses	$ 526	$ 1,163
Conversion of preferred stock to common stock upon initial public offering	$ 239,351	$ —

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

1. NATURE OF THE BUSINESS

Amylyx Pharmaceuticals, Inc., together with its wholly owned subsidiaries ("Amylyx" or the "Company") is a commercial-stage biopharmaceutical company with a goal of improving the quality and length of life of people living with neurodegenerative disease. The Company is focused on the development and potential global commercialization of its product candidate, AMX0035 (sodium phenylbutyrate and taurursodiol) for the treatment of amyotrophic lateral sclerosis ("ALS"). In June 2022, AMX0035 received marketing authorization with conditions as ALBRIOZA by Health Canada for the treatment of ALS, and the Company launched ALBRIOZA in Canada on July 29, 2022. The Company's U.S. New Drug Application ("NDA") for AMX0035 for the treatment of ALS remains under review by the U.S. Food and Drug Administration ("FDA"), and its Marketing Authorisation Application ("MAA") for AMX0035 for the treatment of ALS remains under review by the Committee for Medicinal Products for Human Use ("CHMP") of the European Medicines Agency ("EMA"). The Company is developing AMX0035 for other neurodegenerative diseases by leveraging its unique knowledge and relationships in the neurodegenerative space.

In January 2022, the Company completed an initial public offering (the "IPO"), in which the Company issued and sold 11,369,369 shares of its common stock at a price of $19.00 per share. After deducting underwriting discounts and commissions and estimated offering expenses, the Company received net proceeds of approximately $196.4 million. Upon the completion of IPO, all of the Company's outstanding shares of preferred stock were converted into shares of its common stock.

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 clinical trials, market acceptance and the successful commercialization of ALBRIOZA, which recently received marketing authorization with conditions in Canada, potential difficulties with or delays in timing with respect to the regulatory approval processes of Health Canada, the FDA, the EMA and other comparable foreign authorities, 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 ongoing COVID-19 global pandemic, including potential delays associated with the Company's ongoing and anticipated trials. The COVID-19 pandemic may have an adverse impact on the Company's operations, supply chains and distribution systems or those of its contractors, and increase expenses, including as a result of impacts associated with preventive and precautionary measures that are being taken, such as restrictions on travel and border crossings. The Company and its contractors may experience disruptions in supply of items that are essential for its research and development and commercial activities, including, for example, raw materials and bulk drug substances that the Company imports from Europe and Canada used in the manufacturing of AMX0035, and any future product candidates. In addition, the continued spread of COVID-19 has disrupted global healthcare and healthcare regulatory systems which could divert healthcare resources away from, or materially delay, FDA approval and approval by other health authorities worldwide with respect to AMX0035 and any future product candidates. Furthermore, the Company's clinical trials may be negatively affected by the ongoing COVID-19 outbreak. Site initiation, patient enrollment and patient follow-up visits may be delayed, for example, due to prioritization of hospital resources toward the COVID-19 outbreak, travel restrictions, the inability to access sites for initiation and monitoring, and difficulties recruiting or retaining patients in the Company's ongoing and planned clinical trials.

There can be no assurance that the Company will be able to successfully complete the development of, or receive additional regulatory approval for, any products developed, and if approved, that any products will be commercially viable. Any products resulting from the Company's current research and development efforts will require significant additional research and development, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts will require significant amounts of additional capital, adequate personnel, infrastructure, and extensive compliance reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Going Concern

In accordance with Accounting Standards Update ("ASU") 2014-15, *Disclosure of Uncertainties About an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are conditions and events that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the unaudited consolidated financial statements are issued.

Since its inception, the Company has devoted substantially all of its efforts to research and development and pre-commercialization activities, including recruiting management and technical staff, raising capital, producing materials for non-clinical and clinical studies, and building infrastructure to support such activities. Expenses have primarily been for research and development and related general and administrative costs. The Company has generated revenues through five grants from ALS Association, ALS Finding a Cure Foundation, Cure Alzheimer's Fund, Alzheimer's Drug Discovery Foundation and Alzheimer's Association (collectively, the "Grantors"). In addition to money received from its grants, the Company has also financed its operations through the public offering of its common stock, private sales of preferred stock, and convertible notes.

The accompanying condensed consolidated financial statements have been prepared on a going-concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The Company has incurred recurring losses and negative cash flows from operations since inception. As of June 30, 2022, the Company had an accumulated deficit of $257.8 million. The Company expects its operating losses and negative operating cash flows to continue into the foreseeable future as it launches its first commercial product, ALBRIOZA, which recently received marketing authorization with conditions in Canada, and continues to build capabilities and develop AMX0035, and any future product candidates. In the event that management does not achieve revenue anticipated in its operating plan, management has the ability and commitment to reduce operating expenses as necessary. The Company expects that its cash, cash equivalents and short-term investments as of June 30, 2022 will enable the Company to fund its ongoing operating expenses and capital expenditure requirements for at least the twelve-month period following the issuance of these condensed consolidated financial statements.

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, 2021 and the notes thereto, which are included in the Company's most recent Annual Report on Form 10-K. Since the date of those consolidated financial statements, there have been no material changes to its significant accounting policies, with the exception of significant accounting policies related to the adoption of FASB ASC Topic 842, *Leases*, effective January 1, 2022, as described below.

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 United States of America ("GAAP") and include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and ASU of the Financial Accounting Standards Board ("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. Estimates are used in the following areas, among others: determining the fair value of the Company's common stock prior to the Company's IPO in January 2022; determining the fair value of convertible notes; accrued expenses; stock option valuations; valuation allowance for deferred tax assets and research and development expenses.

Recently Adopted Accounting Pronouncements

Effective January 1, 2022, the Company adopted the requirements under the Financial Accounting Standards Board ("FASB"), Accounting Standards Codification ("ASC") 842, *Leases* ("ASC 842") using the cumulative effect adjustment transition option. Comparative periods have not been restated. This standard requires entities that lease assets to recognize the assets and liabilities for the rights and obligations created by those leases on the balance sheet. The Company elected the available package of practical expedients which allows it to not reassess previous accounting conclusions around whether arrangements are or contain leases, the classification of its leases, and the treatment of initial direct costs. The Company has made an accounting policy election to keep leases with an initial term of 12 months or less off of the balance sheet. ASC 842 was issued in order to increase transparency

and comparability of financial reporting related to leasing arrangements. The main difference between previous GAAP ("ASC 840") and ASC 842 is the recognition of right-of-use lease assets and lease liabilities by lessees for those leases that were classified as operating leases under ASC 840. At January 1, 2022, the Company recorded right-of-use assets of $2.2 million and operating lease liabilities of $2.2 million. Adoption of the standard did not have a material impact on the consolidated statements of operations. For additional information regarding how the Company is accounting for leases under ASC 842, refer to Note 5, Leases.

New Accounting Pronouncements Not Yet Adopted

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326) Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"). The provisions of ASU 2016-13 modify the impairment model to utilize an expected loss methodology in place of the currently used incurred loss methodology and require a consideration of a broader range of reasonable and supportable information to inform credit loss estimates. Credit losses relating to available-for-sale debt securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. ASU 2016-13 requires a cumulative effect adjustment to the consolidated balance sheet as of the beginning of the first reporting period in which the guidance is effective. In November 2019, the FASB issued ASU 2019-10, *Financial Instruments—Credit Losses (Topic 326), Derivatives and Hedging (Topic 815) and Leases (Topic 842): Effective Dates*, which defers the effective date of ASU 2016-13 to fiscal years beginning after December 15, 2022 for all entities except Securities and Exchange Commission filers that are not smaller reporting companies. ASU 2016-13 will be effective for the Company beginning January 1, 2023. The Company intends to adopt the ASU when it becomes effective. The Company is currently evaluating the impact of this ASU and does not expect that adoption of this standard will have a material impact on its condensed consolidated financial statements and related disclosures.

3. SHORT-TERM INVESTMENTS

Short-term investments, which are classified as available-for-sale, consisted of the following:

Balance at June 30, 2022:	Amortized Cost Basis	Unrealized Loss	Fair Value
		(in thousands)	
Treasury notes	$ 6,011	$ (11)	$ 6,000
Treasury bills	47,953	(36)	47,917
Commercial paper	62,835	(1)	62,834
Corporate debt securities	22,643	(153)	22,490
Total short-term investments	$ 139,442	$ (201)	$ 139,241

Balance at December 31, 2021:	Amortized Cost Basis	Unrealized Loss	Fair Value
		(in thousands)	
Commercial paper	$ 33,979	$ (1)	$ 33,978
Corporate debt securities	11,953	(4)	11,949
Total short-term investments	$ 45,932	$ (5)	$ 45,927

As of June 30, 2022 and December 31, 2021, all investments had contractual maturities within one year.

4. FAIR VALUE MEASUREMENTS

The following table presents 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:

	June 30, 2022			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
Assets:				
Money market funds	$ 45,374	$ —	$ —	$ 45,374
Short-term investments:				
Treasury notes	6,000	—	—	6,000
Treasury bills	47,917	—	—	47,917
Commercial paper	—	62,835	—	62,835
Corporate debt securities	—	22,489	—	22,489
Restricted cash	419	—	—	419
Total financial assets	$ 99,710	$ 85,324	$ —	$ 185,034

	December 31, 2021			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
Assets:				
Money market funds	$ 49,271	$ —	$ —	$ 49,271
Short-term investments:				
Commercial paper	—	33,978	—	33,978
Corporate debt securities	—	11,949	—	11,949
Restricted cash	189	—	—	189
Total financial assets	$ 49,460	$ 45,927	$ —	$ 95,387

The Company classifies its money market funds, treasury bills and treasury notes as Level 1 assets under the fair value hierarchy, as these assets have been valued using quoted market prices in active markets without any valuation adjustment. The Company classifies its commercial paper and corporate debt securities 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 that may include trade information, broker or dealer quotes, bids, offers, or a combination of these data sources.

5. LEASES

The Company adopted ASC 842 on January 1, 2022. ASC 842 allows the Company to elect a package of practical expedients, which include: (i) an entity need not reassess whether any expired or existing contracts are or contain leases; (ii) an entity need not reassess the lease classification for any expired or existing leases; and (iii) an entity need not reassess any initial direct costs for any existing leases. Another practical expedient allows the Company to use hindsight in determining the lease term when considering lessee options to extend or terminate the lease and to purchase the underlying asset. The Company has elected to utilize this package of practical expedients and has not elected the hindsight methodology in its implementation of ASC 842.

The Company leases its office facilities under non-cancelable operating leases that expire at various dates through October 2026. The Company entered into a new office space lease at 121 First Street in Cambridge, Massachusetts on January 10, 2022, for 36 months, with an option to extend the lease for 3 years. The Company initially recognized a right-of-use asset of $5.0 million and a lease liability of $5.0 million upon commencement.

Components of lease expense required by ASC 842 are presented below for the three and six months ended June 30, 2022.

	Three Months Ended June 30, 2022	Six Months Ended June 30, 2022
	(in thousands)	(in thousands)
Lease cost		
Operating lease cost	$ 544	$ 1,048
Total lease cost	$ 544	$ 1,048

Lease liabilities are measured by calculating the present value of remaining lease payments under the lease arrangement. Since the rates implicit in our leases are not readily determinable, we use estimated incremental borrowing rates in determining the discount rate used to calculate the present value of remaining lease payments. The incremental borrowing rate is the rate of interest that we would have to pay to borrow, on a collateralized basis, an amount equal to the lease payments over a similar term equal to the lease term in a similar economic environment. The incremental borrowing rate is based on the information available at commencement date. As we have no recent external borrowings, the incremental borrowing is a hypothetical rate based on our understanding of what our credit rating would be and adjusted to reflect a collateralized borrowing.

The Company's leases contain renewal options that can extend the lease for additional years. Because the Company is not reasonably certain to exercise these renewal options, they are not considered in determining the lease terms, and associated potential additional payments are excluded from lease payments. The Company has elected to account for each lease component and its associated non-lease components as a single lease component and has allocated all of the contract consideration across lease components only. The Company has existing net leases in which the non-lease components (e.g. common area maintenance) are paid separately from rent based on actual costs incurred and therefore are not included in the operating lease right-of-use assets and lease liabilities and are reflected as an expense in the period incurred.

The following table summarizes the presentation in the Company's consolidated balance sheet of its operating leases (in thousands):

	As of June 30, 2022
Assets	
Operating lease right-of-use assets	$ 6,369
Liabilities	
Operating lease right-of-use liabilities, current	$ 1,787
Operating lease right-of-use liabilities, net of current portion	5,287
Total operating lease liabilities	$ 7,074

During the six months ended June 30, 2022, the Company made cash payments of $0.4 million for operating leases. Future minimum lease payments under non-cancelable leases as of June 30, 2022, were as detailed below (in thousands):

	As of June 30, 2022
2022 (remaining 6 months)	$ 1,038
2023	2,417
2024	2,478
2025	1,586
2026	476
Total undiscounted lease payments	7,995
Less: imputed interest	(921)
Total operating lease liabilities	$ 7,074

As of June 30, 2022, the weighted average remaining lease term was 3.4 years and the weighted average incremental borrowing rate used to determine the operating lease right-of-use assets was 7.3%.

ASC 840 Disclosures

Future minimum lease payments under non-cancelable leases as of December 31, 2021, were as detailed below (in thousands):

	As of December, 31, 2021
2022	$ 532
2023	541
2024	555
2025	563
2026	476
Total operating lease liabilities	$ 2,667

6. ACCRUED EXPENSES

Accrued expenses consisted of the following:

	June 30, 2022	December 31, 2021
	(in thousands)	
External research and development	$ 4,138	$ 5,666
Payroll and employee related expenses	6,479	4,280
Accrued consulting and other professional fees	6,108	2,820
Other accrued expenses	535	258
Total accrued expenses	$ 17,260	$ 13,024

7. CONVERTIBLE NOTES

Issuance of the 2021 Notes (the "2021 Notes")

In January 2021, the Company issued, in aggregate, $27.3 million in convertible notes ("2021 Notes") to certain investors, including related parties, of which proceeds of $1.2 million were received in advance of issuance of the 2021 Notes in December 2020 and the remaining proceeds of $26.1 million were received in January and February 2021. The 2021 Notes were to mature on June 30, 2022 and carried both automatic and optional conversion features. The 2021 Notes were secured and carried an interest rate of 3%. The Company recorded the $1.2 million of proceeds received in December 2020 as proceeds received in advance of issuance of 2021 Notes in the consolidated balance sheet as of December 31, 2020, as the subscription agreement and commitment to issue the 2021 Notes was not effective until January 2021.

The 2021 Notes contained the following features:

Automatic Conversion Features—The 2021 Notes were to automatically convert into conversion shares ("Conversion Shares") upon (i) an initial public offering, (ii) any transaction in which the Company merges with, consolidates with or enters into other similar transaction with a Special Purpose acquisition Corp ("SPAC"), resulting in some or all of its shares being registered for sale under applicable securities laws and listed for trading on a national or foreign exchange ("De-SPAC transaction"), (iii) the acquisition of the Company by another person or entity by means of any transaction in which holders of the outstanding voting securities of the Company immediately before such transaction held less than 50% of the voting securities of the Company or the surviving corporation after such transaction or a sale of all or substantially all of the assets of the Company but excluding De-SPAC transaction, initial public offering, and the occurrence of equity financing in which the Company sold shares of its preferred stock for new money and which was neither an initial public offering or a Qualified Financing ("Change of Control") and (iv) the closing of a sale of an equity transaction in which the Company sold shares with an aggregate gross proceeds of at least $10.0 million ("Qualified Financing").

In the event of a Change of Control, De-SPAC transaction, or an initial public offering, the Conversion Shares would be common stock of the Company. In the event of a Qualified Financing, the Conversion Shares would be shares of preferred stock issued in such transaction.

Optional Conversion Feature—The holders of the 2021 Notes had the option to elect to convert their notes into Conversion Shares at the Conversion Price upon the occurrence of an equity financing in which the Company sold shares of its preferred stock for new money and which was neither an initial public offering or a Qualified Financing ("Non-Qualified Financing" and together with the initial public offering, De-SPAC transaction, Change of Control, and the Qualified Financing, collectively, the "Conversion

Events"). In the event of a Non-Qualified Financing, the Conversion Shares would be the class of equity shares issued in such transaction. The 2021 Notes would be deemed to have converted into the Conversion Shares if no election was made by the holders of the 2021 Notes.

Conversion Price—Upon the occurrence of an initial public offering, the 2021 Notes would convert into shares of common stock at the conversion price equal to the lesser of (i) 85% of the price at which the Company offered each share of common stock in the initial public offering without deducting any amount for discounts, commissions, fees, or other costs and (ii) $600.0 million divided by the fully diluted capital.

Upon the occurrence of a De-SPAC transaction, the 2021 Notes would convert into shares of common stock at the conversion price equal to the lesser of (i) 85% of the common stock price in the De-SPAC transaction, which would be determined by dividing (x) the total consideration to be paid to common stockholders upon a De-SPAC transaction less the principal amount of the 2021 Notes including accrued and unpaid interest by (y) the common stock issued and outstanding immediately prior to the De-SPAC transaction and that would be exchanged as a result of the De-SPAC transaction including common stock that would be issued upon the exercise of stock options immediately before the Change of Control transaction but excluding the common stock issuable upon conversion of the 2021 Notes and (ii) $600.0 million divided by the fully diluted capital.

Upon the occurrence of a Change of Control, the 2021 Notes would convert into shares of common stock at the conversion price equal to the lesser of (i) 85% of the common stock price in the Change of Control, which would be determined by dividing (x) the total consideration to be paid to common stockholders upon a Change of Control less the principal amount of the 2021 Notes including accrued and unpaid interest by (y) the common stock issued and outstanding immediately prior to the Change of Control and that would be exchanged upon a Change of Control including common stock that would be issued upon the exercise of stock options before the Change of Control but excluding the common stock issuable upon conversion of the 2021 Notes and (ii) $600.0 million divided by the fully diluted capital.

Upon a Qualified Financing, the 2021 Notes would convert into shares of preferred stock issued in the Non-Qualified Financing at the conversion price equal to the lesser of (i) 85% of the lowest price at which the Company sold shares of its stock in the Qualified Financing and (ii) $600.0 million divided by the fully diluted capital.

Repayment—Each holder of the 2021 Notes had the option to elect to receive a payment in the amount equal to the principal amount plus accrued and unpaid interests upon a Change of Control. If a Change of Control occurred and no election was made by the holder, the principal amount and accrued and unpaid interest would be deemed to have automatically be converted into shares of the Company's common stock of the Company immediately prior to the close of the Change of Control.

The Company qualified for and elected to account for the 2021 Notes under the fair value option and, in doing so, bypassed the analysis of potential embedded derivative features. The Company believes that the fair value option better reflects the underlying economics of the 2021 Notes. As a result, the 2021 Notes were recorded at fair value upon issuance, which was determined to be equal to principal amounts of these notes of $27.3 million. At each financial reporting period, and immediately prior to conversion, the Company remeasured the fair value of the 2021 Notes. The change in fair value of the 2021 Notes from inception to June 30, 2021 totaled $5.2 million, which is recorded as change in fair value of convertible notes in the consolidated statement of operations for the six months ended June 30, 2021.

Conversion of the 2021 Notes

In July 2021, the Company consummated a financing transaction in which it issued shares of Series C-1 redeemable convertible preferred stock. The consummation of this financing transaction resulted in the automatic conversion of the 2021 Notes into shares of Series C-2 redeemable convertible preferred stock (together with the Series C-1 redeemable convertible preferred stock, the "Series C Preferred Stock") pursuant to their original terms. The Series C Preferred Stock was determined to have a fair value of $10.265809. Under the fair value option, the 2021 Notes were remeasured to fair value immediately prior to conversion at a price per share equal to the fair value of the Series C-1 redeemable convertible preferred stock. The Company recorded $5.2 million loss related to change in fair value of the 2021 Notes in its consolidated statement of operations for the year ended December 31, 2021. The 2021 Notes converted into 3,170,585 shares of Series C-2 redeemable convertible preferred stock at the effective conversion price of $8.725938.

There were no convertible notes outstanding as of June 30, 2022 or December 31, 2021.

Convertible Notes—Related Parties

There were no convertible notes issued to related parties that were outstanding as of June 30, 2022 or December 31, 2021. In connection with the issuance of the 2021 Notes, the Company issued, in aggregate, $14.3 million of convertible notes to certain related parties. These notes were issued under the same terms and conditions as the 2021 Notes.

Valuation of the 2021 Notes

At the issuance date of the 2021 Notes, the Company determined that the fair value of the 2021 Notes approximated the principal amounts of the 2021 Notes as the transaction was deemed to be at arm's length. Subsequent measurement of fair value of the 2021 Notes at each reporting period was estimated based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The Company used a scenario-based analysis to incorporate estimates and assumptions concerning the Company's prospects and market indications into a model to estimate the value of the 2021 Notes. The most significant estimates and assumptions used as inputs were those concerning timing, probability of possible scenarios for conversion or settlement of the 2021 Notes and discount rates. The fair value of the 2021 Notes upon settlement in July 2021 was determined based on the fair value of the Series C-1 redeemable convertible preferred stock issued. This method was selected as the Company concluded that the contemporaneous financing transaction was an arm's length transaction. The issuance of the Series C-1 redeemable convertible preferred stock was considered to be a Qualified Financing (see Note 8) pursuant to the original terms of the 2021 Notes. Accordingly, the fair value calculation for the 2021 Notes immediately before conversion considered both the fair value of the Series C-1 redeemable convertible preferred stock and the conversion price, which was 85% of the fair value of the Series C-1 redeemable convertible preferred stock. The fair value of the 2021 Notes as of June 30, 2021 was determined to be the same as that on the settlement date in July 2021 based on management's determination of no material changes to the assumptions underlying the determination of the fair value of the 2021 Notes.

8. REDEEMABLE CONVERTIBLE PREFERRED STOCK

On July 1, 2021, the Company amended its certificate of incorporation in which it authorized 13,150,430 shares of Series C-1 redeemable convertible preferred stock and 3,170,585 shares of Series C-2 redeemable convertible preferred stock.

In July 2021, the Company consummated a financing transaction in which it issued 13,150,430 shares of Series C-1 redeemable convertible preferred stock. In connection with the issuance of these shares, the principal including accrued interest of the 2021 Notes totaling $27.7 million automatically converted into 3,170,585 shares of Series C-2 redeemable convertible preferred stock.

The Company's redeemable convertible preferred stock consisted of the following:

	December 31, 2021 (dollars in thousands)				
	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A preferred stock	6,289,609	6,289,609	$ 7,675	$ 7,730	6,407,256
Series B preferred stock	15,100,000	14,496,835	$ 64,387	$ 246,070	16,746,059
Series C-1 preferred stock	13,150,430	13,150,430	$ 134,791	$ 135,000	13,150,430
Series C-2 preferred stock	3,170,585	3,170,585	$ 32,498	$ 27,666	3,170,585
	37,710,624	37,107,459	$ 239,351	$ 416,466	39,474,330

As of December 31, 2021, the holders of the Series C Preferred Stock (together with the "Series A redeemable convertible preferred stock" and "Series B redeemable convertible preferred stock", collectively, the "Preferred Stock") have the following rights and preferences:

Conversion— On June 18, 2020, in connection with the conversion of the 2017 Notes, the Company adjusted the conversion price for the Series A redeemable convertible preferred stock of $1.229073 per share to $1.2065. The adjustment was made in accordance with the anti-dilution provisions in the certificate of incorporation then in effect immediately prior to the conversion of the 2017 Notes. The adjustment to the conversion price resulted in neither modification nor extinguishment of the Series A redeemable convertible preferred stock as the terms of the Series A redeemable convertible preferred stock were not amended. The adjustment to the conversion price resulted in additional 117,650 shares of common stock to be issued to holders of the Series A redeemable convertible preferred stock upon conversion of such shares into common stock. As of December 31, 2020, these additional shares of common stock were not issued and outstanding.

In July 2021, in connection with the conversion of the 2021 Notes, the Company adjusted the conversion price for the Series B redeemable convertible preferred stock of $16.974077 per share to $14.6942. The adjustment was made in accordance with the anti-dilution provisions in the certificate of incorporation then in effect immediately prior to the conversion of the 2021 Notes. The adjustment to the conversion price resulted in additional 2,249,224 shares of common stock into which Series B redeemable convertible preferred stock would be convertible. As of December 31, 2021, these additional shares were not issued and outstanding. Each share of Preferred Stock is convertible into an equivalent number of common stock, at any time, at the option of the holder. The initial conversion price for the Series C-1 redeemable convertible preferred stock and Series C-2 redeemable convertible preferred stock is the respective original issue prices.

The conversion price for the Preferred Stock was subject to adjustments for stock splits, stock dividends, or similar recapitalization, and subject to adjustments in accordance with the anti-dilution provisions.

The shares of Preferred Stock were to automatically convert into common stock of the Company immediately upon either (a) the closing of the sale of shares of common stock to the public in a firm commitment underwritten public offering pursuant to an effective registration statement under the Securities Act, resulting in at least $75.0 million of proceeds, net of the underwriting discount and commissions, to the Company (a “Qualified IPO”) or (b) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of a majority of the then outstanding shares of Preferred Stock.

Dividends—Dividends may be paid to the holders of the Series A redeemable convertible preferred stock. The holders the Series A redeemable convertible preferred stock are entitled to receive non-cumulative dividends at a rate per annum of $0.073744 per share when and if declared by the Board of Directors. The holders of the Series B redeemable convertible preferred stock were entitled to receive a non-cumulative dividend at the rate of 6% per annum of the Series B original issue price per share when and if declared by the Board of Directors. As of December 31, 2021, no cash dividends were declared or paid. From and after the date of issuance of the Series C Preferred Stock, the Company was not to set, declare, pay or set aside unless holders of the Series C Preferred Stock then outstanding shall first receive, or simultaneously receive, dividends on each outstanding share of Series C Preferred Stock in an amount equal to (i) in the case of dividends being distributed to common stock or any class or series of capital stock that is convertible into common stock, the equivalent dividend on an as-converted basis or (ii) in the case of dividends being distributed on a series or class not convertible into common stock, an additional dividend equal to a dividend rate calculated based on the respective original issue price of the Series C Preferred Stock. The original issue prices per share for the Series C-1 redeemable convertible preferred stock and Series C-2 redeemable convertible preferred stock were $10.265809 and $8.725938, respectively.

Voting Rights— The holders of the Preferred Stock were entitled to vote on any matter presented to stockholders of the Company for consideration. Each holder of the Preferred Stock was entitled to cast the number of votes equal to the number of shares of common stock into which the shares of the Preferred Stock held by such holder were convertible on such date.

Redemption—The Preferred Stock did not contain any mandatory redemption features. In accordance with FASB ASC Topic 480, Distinguishing Liabilities from Equity (ASC 480), preferred stock issued with redemption provisions that are outside of the control of the Company or that contain certain redemption rights in a deemed liquidation event is required to be presented outside of stockholders’ deficit on the face of the consolidated balance sheets. The Company classified the Preferred Stock outside of the stockholders’ deficit as mezzanine equity because in the event of certain deemed liquidation events, which included events such as a sale or merger, that were not solely within the control of the Company, the shares of the Preferred Stock would have become redeemable at the option of the holders. As of June 30, 2021, the Company did not adjust the carrying values of the Preferred Stock to the redemption values of such shares since a deemed liquidation event did not occur and the shares were not probable of becoming redeemable in the future as of the consolidated balance sheet dates.

Liquidation—In the event of a liquidation, deemed liquidation, dissolution or winding up of the Company, holders of the Preferred Stock would have been entitled to be paid out of the assets of the Company that were available for distribution before any payment is made to the holders of common stock. The amount to paid would have been the greater of (i) respective original issue prices plus any dividends declared but unpaid or (ii) the amount that would have been payable had all shares of Preferred Stock been converted into common stock immediately before such event. If upon any such liquidation, deemed liquidation, dissolution or winding up of the Company, the assets of the Company available for distribution to its stockholders would have been insufficient to pay the holders of Preferred Stock the full amount to which they would have been entitled, the holders of Preferred Stock would have shared ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

After the payment of all preferential amounts required to be paid to the holders of Preferred Stock, the remaining assets of the Company available for distribution to its stockholders would have been distributed among the holders of the shares of common stock on a pro rata basis based on the number of shares held by each such holder.

In January 2022, upon the completion of the initial public offering, all of the Company's outstanding shares of preferred stock were converted into shares of its common stock. There were no redeemable convertible preferred stock outstanding as of June 30, 2022.

9. STOCKHOLDERS' EQUITY (DEFICIT)

Common Stock

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders provided, however, that, except as otherwise required by law, holders of common stock shall not be entitled to vote on any amendment to the Corporation's Certificate of Incorporation that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the Certificate of Incorporation or pursuant to the Delaware General Corporation Law. Common stockholders are entitled to receive dividends, as may be declared by the Company's Board of Directors, if any, subject to the preferential dividend rights of the Preferred Stock. No dividends have been declared or paid during the six months ended June 30, 2022 and 2021.

The Company had reserved shares of common stock for issuance in connection with the following:

	June 30, 2022	December 31, 2021
Common stock authorized	300,000,000	56,500,000
Common stock issued and outstanding	58,533,226	7,020,487
Common stock authorized and reserved for future issuances:		
Series A redeemable convertible preferred stock	—	6,407,256
Series B redeemable convertible preferred stock	—	16,746,059
Series C-1 redeemable convertible preferred stock	—	13,150,430
Series C-2 redeemable convertible preferred stock	—	3,170,585
Common stock reserved for the exercise of stock options	8,528,039	5,339,011
Common stock reserved for the unvested restricted stock units	670,013	—
Common stock reserved for future issuance of share-based awards	3,121,919	1,444,492
Total common stock authorized and reserved for future issuance	12,319,971	46,257,833
Unreserved common stock available for future issuance	229,146,803	3,221,680

10. STOCK OPTION AND GRANT PLAN

Stock Incentive Plan

In January 2022, the Company's board of directors adopted, and its stockholders approved the 2022 Stock option and Incentive Plan (the "2022 Plan"), which became effective on January 5, 2022, at which point no further grants would be made under the 2015 Stock Option and Restricted Stock Plan (the "2015 Plan"). Under the 2022 Plan, the Company may grant incentive stock options ("ISOs"), non-statutory stock options, stock appreciation rights, restricted stock units, restricted stock awards and other stock-based awards. As of June 30, 2022, there were 3,121,919 shares available for future issuance under the 2022 Plan. The options issued under the 2022 Plan expire after 10 years.

Initially, subject to adjustment as provided in the 2022 Plan, the aggregate number of shares of the Company's common stock available for issuance under the 2022 Plan is 7,650,000. The number of shares of the Company's common stock reserved for issuance under the 2022 Plan will automatically increase on January 1 of each year commencing January 1, 2023, by 5% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by the Company's board of directors. The maximum current number of shares that may be issued pursuant to the exercise of ISOs under the 2022 Plan is 7,650,000.

The maximum number of shares of the Company's common stock subject to awards granted under the 2022 Plan or otherwise during a single calendar year to any individual nonemployee director, taken together with any cash fees paid by the Company to such nonemployee director during the calendar year for serving on the Company's board of directors, will not exceed

$750,000 in total value, or, with respect to the calendar year in which a nonemployee director is first appointed or elected to the Company's board of directors, $1,000,000.

All options and awards granted under the 2015 Plan consisted of the Company's common stock. As of January 6, 2022, no additional stock awards have been or will be granted under the 2015 Plan. Although the 2015 Plan was terminated as to future awards in January 2022, it continues to govern the terms of options that remain outstanding under the 2015 Plan.

General Option Information

A summary of option activity for the six months ended June 30, 2022, 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, 2021	5,339,011	$ 5.54	8.7	$ 15,627
Granted	3,872,520	$ 19.07		
Exercised	(669,040)	$ 0.37		$ 5,961
Cancelled or forfeited	(14,452)	$ 14.07		
Outstanding at June 30, 2022	8,528,039	$ 12.08	8.8	$ 65,275
Options exercisable as of June 30, 2022	1,393,475	$ 4.71	7.4	$ 20,276
Options unvested as of June 30, 2022	7,134,564	$ 13.52	9.1	$ 44,999
Weighted average grant-date fair value of options granted during the period	$ 14.20			

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of options exercised during the six months ended June 30, 2022 and 2021 was $6.0 million and $0.1 million, respectively.

The total fair value of stock options vested during the six months ended June 30, 2022 and 2021 was $3.2 million and $0.4 million, respectively.

Restricted Stock Unit Activity

A summary of restricted stock unit activity for the six months ended June 30, 2022, is as follows:

	Number of shares	Weighted Average Grant Date Fair Value
Nonvested as of December 31, 2021	—	$ —
Granted	672,313	$ 18.41
Vested	—	—
Forfeited	(2,300)	$ 16.75
Nonvested as of June 30, 2022	670,013	$ 18.42

Summary of Stock-Based Compensation Expense

Stock-based compensation expense recorded in the condensed consolidated statements of comprehensive income for the three and six months ended June 30, 2022 and 2021, is as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
	(in thousands)		(in thousands)	
Research and development	$ 1,460	$ 187	$ 2,585	$ 391
General and administrative	4,247	470	7,514	852
Total stock-based compensation expense	$ 5,707	$ 657	$ 10,099	$ 1,243

The following table summarizes stock-based compensation by type of award:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
	(in thousands)		(in thousands)	
Stock options	$ 4,955	$ 657	$ 8,863	$ 1,243
Restricted stock units	752	—	1,236	—
Total stock-based compensation expense	$ 5,707	$ 657	$ 10,099	$ 1,243

The following table summarizes unrecognized stock-based compensation expense as of June 30, 2022, by type of awards, and the weighted-average period over which that expense is expected to be recognized. The total unrecognized stock-based compensation expense will be adjusted for actual forfeitures as they occur.

	As of June 30, 2022	
	Unrecognized Expense	Weighted-average Recognition Period
	(in thousands)	(in years)
Stock options	$ 66,408	3.18
Restricted stock units	$ 11,103	3.62

11. NET LOSS PER SHARE

Net Loss per Share Attributable to Common Stockholders—Because the Company reports a net loss attributable to common stockholders, basic and diluted net loss per share attributable to common stockholders are the same for both periods presented. All preferred stock, stock options and restricted stock units have been excluded from the computation of diluted weighted-average shares outstanding because such securities would have an antidilutive impact. The following common stock equivalents outstanding at each period end have been excluded from the calculation of diluted net loss per share because their inclusion would have been antidilutive:

	June 30, 2022	December 31, 2021
Options to purchase common stock	8,528,039	5,339,011
Unvested restricted stock units	670,013	—
Redeemable convertible preferred stock	—	39,474,330

12. RELATED PARTY TRANSACTIONS

Convertible Notes

In connection with the issuance of the 2021 Notes, the Company issued, in aggregate, $14.3 million of convertible promissory notes to Morningside Ventures Investments Limited, and certain members of the board of directors of the Company. Morningside Ventures Investments Limited is a 5% significant stockholder and has appointed representatives to the board of directors of the Company. These notes were issued under the same terms and conditions as the 2021 Notes (see Note 7).

Supplier Agreements

In the ordinary course of business, the Company may purchase materials or supplies or services from entities that are associated with a party that meets the criteria of a related party of the Company. These transactions are reviewed quarterly and to date have not been material to the Company's condensed consolidated financial statements.

13. COMMITMENTS AND CONTINGENCIES

Lease Commitments

The Company has two operating lease agreements for its office space. See Note 5, *Leases*, to these notes to condensed consolidated financial statements for additional information.

Letter of Credit

Restricted cash consists of cash serving of $0.2 million as collateral for a letter of credit issued for the Company's office space, and $0.2 million as collateral for a corporate credit card program. As of June 30, 2022 and December 31, 2021, the Company's restricted cash balance was $0.4 million and $0.2 million on its consolidated balance sheets, respectively.

Legal Proceedings

The Company is not currently a party to any material legal proceedings. At each reporting date, the Company evaluates whether or not a potential loss amount or potential range of loss is probable and reasonably estimated under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company recognizes expenses for its costs related to its legal proceedings, as incurred.

Royalty Payments

Between August 2016 and February 2019, the Company entered into agreements with the Grantors. Under the terms of the agreements, the Company was granted, in aggregate, $4.3 million. These grants were provided to the Company for the purpose of furthering the research and development of AMX0035 as a therapeutic benefit for ALS disease and Alzheimer's diseases. Under the terms of the arrangements, the Company would receive a tranche of funds as it completes certain milestones. Pursuant to the terms of the grant agreements, the Company has certain payment obligations that are contingent upon future events such as the achievement of commercialization or the receipt of proceeds from a revenue generating transaction resulting from the projects for which the grants are used for.

Pursuant to the terms of the respective grant agreements among the Company, ALS Association and ALS Finding a Cure, the Company will be required to make royalty payments to each Grantor in the total amount equal to 150% of the grant received. The royalty payments will be achieved through a combination of the following payment methods: (i) an annual installment payment of 3% of net sales of any products developed under the project for which the grant was used for and (ii) 3% of cash proceeds resulting from revenue generating transaction under the project for which the grants are used for.

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). The royalty payment will be made through a combination of the following payment methods: (i) 4% of annual net sales of any product commercialized from the project for which the grant was used for and directly related to the treatment of the Alzheimer's disease and (ii) 15% of all royalties and cash proceeds resulting from revenue generating transactions associated with the projects for which the grants were used for under the grant agreements.

As the conditions that would trigger the future royalty payments have not occurred, no amounts have been recorded in the condensed consolidated financial statements as of June 30, 2022 and December 31, 2021.

14. SUBSEQUENT EVENTS

The Company has evaluated all subsequent events after June 30, 2022 and through the date of this filing, and there were no material subsequent events requiring disclosure.

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 factors set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2021, 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

Our mission is to develop therapies that change the treatment paradigm for amyotrophic lateral sclerosis ("ALS"), and a broad range of other neurodegenerative diseases by keeping neurons alive. Unlike most other cells in the body that regularly die and are replaced as part of healthy function, mature neurons are normally resistant to cell death and generally cannot regenerate. The Company is focused on the development and potential global commercialization of its product candidate, AMX0035 (sodium phenylbutyrate and taurursodiol) for the treatment of ALS. In June 2022, AMX0035 received marketing authorization with conditions as ALBRIOZA by Health Canada for the treatment of ALS. We commenced Canadian commercial sales of ALBRIOZA in the third quarter of 2022.

Our U.S. New Drug Application ("NDA"), for AMX0035 (sodium phenylbutyrate and taurursodiol) for the treatment ALS remains under review by the U.S. Food and Drug Administration ("FDA"). The Prescription Drug User Fee Act date, the target date by which the FDA intends to complete its review and take action on the U.S. NDA for AMX0035, is September 29, 2022, which was extended from June 29, 2022, to allow more time for the FDA to review additional analyses of data from the Company's clinical studies. On March 30, 2022, the FDA held a virtual meeting of its Peripheral and Central Nervous System Drugs Advisory Committee, ("Advisory Committee"). At that meeting, on the question whether the data from our randomized, controlled Phase 2 CENTAUR trial and open-label extension ("OLE") trial established a conclusion that AMX0035 is effective in the treatment of patients with ALS, the Advisory Committee voted 4 (yes) and 6 (no). In July 2022, the FDA informed us, and formally announced in the Federal Register on August 3, 2022, that it will reconvene the Advisory Committee on September 7, 2022. Although the FDA considers the recommendations of its advisory committees, the recommendation by the Advisory Committee is non-binding. The final decision regarding approval of a pending NDA is made by the FDA, and we remain committed to pursuing its approval given the pressing need for new treatments for ALS. We believe AMX0035 has the potential to be a foundational therapy, meaning that it could be used alone or in conjunction with other therapies to change the treatment paradigm across a broad range of neurodegenerative diseases.

Our Marketing Authorisation Application ("MAA") also remains under review by the Committee for Medicinal Products for Human Use ("CHMP") of the European Medicines Agency ("EMA"). The review process is proceeding as expected, with receipt of the Day 120 List of Questions following the June CHMP meeting. We will continue to work with EMA through its review process, and the Company expects a decision in the first half of 2023.

AMX0035 is a dual UPR-Bax apoptosis inhibitor composed of sodium phenylbutyrate and TURSO (also known as tauroursodeoxycholic acid ("TUDCA")). Through the resolution of the unfolded protein response ("UPR") and by inhibiting translocation of the Bcl-2 Associated X-protein ("Bax") to the outer mitochondrial membrane, we have shown in multiple models that AMX0035 can keep neurons alive under a variety of different conditions and stresses, including in in vitro models of neurodegeneration, endoplasmic reticulum stress, mitochondrial dysfunction, oxidative stress and disease-specific models of a variety of other conditions, as well as in vivo models of Alzheimer's Disease ("AD") and multiple sclerosis. We are pursuing ALS as our first indication as it is a disease of rapid and profound neurodegeneration, and we are focused on the development and potential commercialization of AMX0035 for ALS globally.

In November 2021, we initiated a global Phase 3 clinical trial of AMX0035 for the treatment of ALS, known as the PHOENIX trial, at clinical trial sites in the U.S. and Europe. Enrollment in this trial has completed in the U.S. and remains ongoing in Europe. We anticipate topline results from the PHOENIX trial in 2024. This trial is designed to provide further data supporting the safety and efficacy of AMX0035 for the treatment of ALS and to further support our global regulatory efforts. In July 2022, we announced a planned OLE phase for the PHOENIX trial. In March 2022, we announced the launch of a U.S. expanded access program ("EAP") that the FDA has authorized for people with ALS who meet eligibility criteria for participation. The U.S. EAP for AMX0035 is running in parallel with the PHOENIX trial. People living with ALS who are eligible for PHOENIX are not eligible for the U.S. EAP as the criteria for entry do not overlap. Based on dialogue with the FDA prior to our NDA submission, including at a

pre-NDA meeting recommended by the FDA and subsequent discussions, we believed that data from the PHOENIX trial would not be required for the FDA to make a determination on the approval of AMX0035 for the treatment of ALS, although we had no assurance that the FDA would not require further data before making a determination. In July 2022, the FDA informed us, and formally announced in the Federal Register on August 3, 2022, that it will reconvene the Advisory Committee to further discuss the NDA for AMX0035 on September 7, 2022 to focus on an additional analysis of data from our clinical trials that were determined by the FDA to constitute a major amendment to the NDA. Based on the outcome of this planned Advisory Committee meeting and the Advisory Committee meeting held on March 30, 2022, it remains uncertain whether additional clinical data will be required to make a determination on the approval of AMX0035 for the treatment of ALS.

Since inception in 2013, we have devoted substantially all of our efforts to research and development and pre-commercialization activities, including recruiting management and technical staff, raising capital, producing materials for non-clinical and clinical studies, planning for potential commercialization and building infrastructure to support such activities. Other than ALBRIOZA in Canada, which received marketing authorization with conditions from Health Canada, we do not have any products approved for sale and as of June 30, 2022, we have not generated any revenue from product sales. As of June 30, 2022, we have funded our operations primarily through the public offering of our common stock, private sales of preferred stock, and convertible notes. We have also generated grant revenues through five grants from ALS Association, ALS Finding a Cure Foundation, Cure Alzheimer's Fund, Alzheimer's Drug Discovery Foundation and Alzheimer's Association (the "Grantors").

We have incurred operating losses since inception, including a net loss of $101.9 million for the six months ended June 30, 2022. As of June 30, 2022, we had an accumulated deficit of $257.8 million. These losses have resulted primarily from costs incurred in connection with research and development activities and general and administrative costs associated with our operations. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future as we advance AMX0035 and any future product candidates through preclinical and clinical development, hire additional clinical, scientific, management and administrative personnel, seek regulatory approval and pursue commercialization of any approved product candidates. To date, we have primarily developed AMX0035 internally, with assistance from our network of contract research organizations ("CROs") and other advisors. This has resulted in increased research and development spending but has enabled us to manage AMX0035 efficiently through the development and manufacturing process.

We expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company. As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through 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 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, however, that our current operating plan will be achieved or that additional funding will be available on terms acceptable to us, or at all.

As of June 30, 2022, we had cash, cash equivalents and short-term investments of $206.7 million. We believe that our existing cash, cash equivalents and short-term investments will be sufficient to meet our anticipated operating and capital expenditure requirements for at least one year from the date of this filing. 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—Funding Requirements" below.

Impact of COVID-19

The development of AMX0035 and any future product candidates could be disrupted and materially adversely affected in the future by COVID-19 pandemic or any future pandemic or calamity. The spread of COVID-19 and identification of new variants of the virus has impacted the global economy and our operations, including requiring us to make certain alterations to our preclinical and clinical trial activities, such as scheduling certain work off-site and performing off-site assessments. In addition, we had to amend our CENTAUR trial protocol to allow for remote visits by patients, instead of patients making site visits and in certain cases we were forced to delay enrollment at certain sites in our Phase 2 clinical trial for AMX0035 in AD.

If the disruption due to the ongoing COVID-19 pandemic continues, our ongoing global Phase 3 PHOENIX clinical trial for AMX0035 for the treatment of ALS could be delayed due to government orders and site policies on account of the pandemic. Additionally, some patients may be unwilling or unable to travel to study sites, enroll in our trials or be unable to comply with clinical trial protocols, which would delay our ability to conduct preclinical studies and clinical trials or release clinical trial results, as well as delay our ability to obtain regulatory approval for and commercialize AMX0035. Furthermore, the COVID-19 pandemic could continue to affect our employees or the employees of research sites and service providers on whom we rely as well as those of

companies with which we do business, including our suppliers, thereby disrupting our business operations. Existing or renewed quarantines and travel restrictions imposed by governments in the jurisdictions in which we and the companies with which we do business operate could materially impact the ability of employees to access preclinical and clinical sites, laboratories, manufacturing sites and offices. We have implemented and continue to follow work-at-home policies and may experience limitations in employee resources. Our continued reliance on personnel working from home may negatively impact productivity, or disrupt, delay or otherwise adversely impact our business.

Components of Our Results of Operations

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the research and development of AMX0035. We expense research and development costs as incurred. These expenses include:

- expenses incurred under agreements with CROs, contract manufacturing organizations ("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, 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 substantially in connection with our planned clinical development activities in the near term and in the future and to fund commercialization activities in Canada and any other jurisdictions in which AMX0035 is approved. 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 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 research studies of our current or future product candidates; and
- the number of product candidates we are developing.

The successful development and commercialization of AMX0035 and any 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 nonclinical and clinical development activities;
- the number and scope of nonclinical and clinical trials for separate indications we decide to pursue;
- raising necessary additional funds;
- 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 Health Canada, the U.S. FDA or the EMA, or any other comparable foreign regulatory authority;
- the receipt and related terms of regulatory approvals from applicable regulatory authorities, including our marketing authorization with conditions from Health Canada for ALBRIOZA;
- the availability of drug substance and drug product for use in production of AMX0035;
- establishing and maintaining agreements with third-party manufacturers for clinical supply for our clinical trials and commercial manufacturing, if AMX0035 is approved;
- our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- our ability to protect our rights in our intellectual property portfolio;
- the commercialization in Canada of AMX0035 (known as ALBRIOZA in Canada) and in other potential jurisdictions, if and when approved;
- obtaining and maintaining third-party insurance coverage and adequate reimbursement;
- the acceptance of AMX0035, if approved, by patients, the medical community and third-party payors;
- competition with other product; 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 or any 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 regulatory approval for AMX0035 or any future product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs for personnel in executive, finance, sales, marketing, as well as administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters; professional fees for accounting, auditing, tax and administrative consulting services; insurance costs; administrative travel expenses; marketing expenses; rent expense and other operating costs. We anticipate that our general and

administrative expenses will continue to increase in the future as we further increase our headcount to support our continued research activities and development of AMX0035 and as we continue to increase headcount and incur other significant costs related to our pre-commercialization activities as we prepare for potential near term regulatory approvals. We also anticipate that we will continue to incur increased accounting, audit, legal, regulatory, compliance, and director and officer insurance costs as well as investor and public relations expenses associated with being a public company. We have received marketing authorization with conditions for ALBRIOZA for the treatment of ALS in Canada and are pursuing regulatory approval of AMX0035 for the treatment of ALS in the United States and Europe. As we implement our commercialization plans in Canada and prepare for a potential approval in the United States and Europe, we have been incurring a substantial increase, and anticipate further increases in, payroll and expense as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of AMX0035.

Other Income (Expense), Net

Interest Income

Interest income consists of interest income earned on our cash and cash equivalents, and short-term investments.

Other (Expense) Income, Net

Other (expense) income, net consists primarily of realized and unrealized gains and losses on foreign exchange transactions.

Change in Fair Value of Convertible Notes

Change in fair value of convertible notes is comprised of adjustments to the fair value of our 2021 Notes. As permitted under ASC Topic 825, *Financial Instruments* (ASC 825), we elected the fair value option to account for our 2021 Notes, and as a result, we measured our 2021 Notes at fair value at each financial reporting period and immediately before conversion in July 2021. All changes to the fair value of our 2021 Notes for the six months ended June 30, 2021 resulted in a loss. Our 2021 Notes converted into shares of Series C-2 redeemable convertible preferred stock concurrently with the issuance of our Series C-1 redeemable convertible preferred stock. Immediately prior to the conversion, we determined the fair value of our 2021 Notes based on the fair value of the Series C-1 redeemable convertible preferred stock and the conversion price at which these notes converted, which was at 85% of the fair value of the Series C-1 redeemable convertible preferred stock.

Income Taxes

The provision for income taxes primarily consists of provisions for foreign taxes payable.

Results of Operations

Comparison of the three months ended June 30, 2022 and 2021

The following table summarizes our results of operations for the periods presented:

	Three Months Ended June 30,			
	2022	2021	$ Change	% Change
	(in thousands)			
Operating expenses:				
Research and development	$ 24,259	$ 10,929	$ 13,330	122 %
General and administrative	29,994	7,658	22,336	292 %
Total operating expenses	54,253	18,587	35,666	192 %
Loss from operations	$ (54,253)	$ (18,587)	$ (35,666)	192 %
Other income (expense), net:				
Interest income	402	1	401	40,128 %
Change in fair value of convertible notes	—	(3,310)	3,310	(100)%
Other (expense), net	(42)	(26)	(16)	63 %
Other income (expense), net	360	(3,335)	3,695	(111)%
Loss before income taxes	(53,893)	(21,922)	(31,971)	146 %
Provision for income taxes	174	—	174	*NM
Net loss	$ 54,067	$ 21,922	$ 32,145	147 %

* NM - not meaningful

Research and Development Expenses

The following table summarizes our research and development expenses for the three months ended June 30, 2022 and 2021:

	Three months ended June 30,			
	2022	2021	$ Change	% Change
	(in thousands)			
AMX0035 – ALS	$ 15,484	$ 4,107	$ 11,377	277 %
Payroll and personnel-related	6,650	1,741	4,909	282 %
Other	2,125	5,081	(2,956)	(58)%
	$ 24,259	$ 10,929	$ 13,330	122 %

Research and development expenses were $24.3 million for the three months ended June 30, 2022, compared to $10.9 million for the three months ended June 30, 2021. During these periods, all our research and development expenses were related to the development of and clinical trials of AMX0035. The increase of $13.3 million was primarily due to a $11.4 million increase in spending on AMX0035 for the treatment of ALS, a $4.9 million increase in payroll and personnel-related costs, and a $3.0 million decrease in all other costs. The increases in spending on AMX0035 were primarily related to costs associated with our global Phase 3 PHOENIX trial of AMX0035 in ALS that was initiated in November 2021 and consulting and manufacturing development expenses in anticipation of potential commercialization. The increase in payroll and personnel-related costs was primarily due to an increase in the number of employees supporting research and development efforts. Decrease in other costs is primarily due to a decrease in costs associated with research and development for AMX0035 in other indications.

General and Administrative Expenses

General and administrative expenses were $30.0 million for the three months ended June 30, 2022 compared to $7.7 million for the three months ended June 30, 2021. The increase of $22.3 million was primarily due to increases of $14.4 million in payroll and personnel-related costs, including stock-based compensation, $2.9 million in consulting expense, $2.1 million in professional services and $1.3 million in insurance expense. The increase in payroll and personnel-related costs was primarily due to hiring additional personnel in commercial and general and administrative functions to support our growth, as well as commercialization and launch preparation initiatives. The increase in professional services, consulting expenses and insurance expenses were primarily due to an increase in spending for commercial readiness activities and operations as a public company.

Other Income (Expense), Net

Interest Income

Interest income for the three months ended June 30, 2022 was $0.4 million compared to less than $0.1 million for the three months ended June 30, 2021. The increase is primarily attributable to higher investment balances driven by our proceeds received from our January 2022 initial public offering, resulting in higher interest earned.

Change in Fair Value of Convertible Notes

The change in fair value of convertible notes was zero for the three months ended June 30, 2022 due to conversion to preferred stock in July 2021, compared to $3.3 million for the three months ended June 30, 2021. The $3.3 million recorded for the three months ended June 30, 2021 represented a loss in fair value related to our 2021 Notes.

Other (Expense), Net

Other expense, net was less than $0.1 million for the three months ended June 30, 2022 and 2021. The other expense, net in the three months ended June 30, 2022 and 2021 was primarily related to realized and unrealized gains and losses.

Income Taxes

Provision for income taxes was $0.2 million for the three months ended June 30, 2022 compared to zero for the three months ended June 30, 2021, and consists of current income tax expense arising from activities of our foreign subsidiaries.

Comparison of the six months ended June 30, 2022 and 2021

The following table summarizes our results of operations for the periods presented:

	Six Months Ended June 30,			
	2022	2021	$ Change	% Change
	(in thousands)			
Operating expenses:				
Research and development	$ 45,723	$ 17,793	$ 27,930	157 %
General and administrative	56,344	13,662	42,682	312 %
Total operating expenses	102,067	31,455	70,612	224 %
Loss from operations	$ (102,067)	$ (31,455)	$ (70,612)	224 %
Other income (expense), net:				
Interest income	533	3	530	17,677 %
Change in fair value of convertible notes	—	(5,228)	5,228	(100)%
Other (expense) income, net	(61)	235	(296)	(126)%
Other income (expense), net	472	(4,990)	5,462	(109)%
Loss before income taxes	(101,595)	(36,445)	(65,150)	179 %
Provision for income taxes	320	—	320	*NM
Net loss	$ 101,915	$ 36,445	$ 65,470	180 %

* NM – not meaningful

Research and Development Expenses

The following table summarizes our research and development expenses for the six months ended June 30, 2022 and 2021:

	Six months ended June 30,			
	2022	2021	$ Change	% Change
	(in thousands)			
AMX0035 – ALS	$ 30,720	$ 6,376	$ 24,344	382 %
Payroll and personnel-related	12,515	3,189	9,326	292 %
Other	2,488	8,228	(5,740)	(70)%
	$ 45,723	$ 17,793	$ 27,930	157 %

Research and development expenses were $45.7 million for the six months ended June 30, 2022, compared to $17.8 million for the six months ended June 30, 2021. During these periods, all of our research and development expenses were related to the development of and clinical trials of AMX0035. The increase of $27.9 million was primarily due to a $24.3 million increase in spending on AMX0035 for the treatment of ALS, a $9.3 million increase in payroll and personnel-related costs, and a $5.7 million decrease in all other costs. The increases in spending on AMX0035 were primarily related to costs associated with our global Phase 3 PHOENIX trial of AMX0035 in ALS that was initiated in November 2021 and consulting and manufacturing development expenses in anticipation of potential commercialization. The increase in payroll and personnel-related costs was primarily due to an increase in the number of employees supporting research and development efforts. Decrease in other costs were primarily due to a decrease in costs associated with research and development spend for AMX0035 in other indications.

General and Administrative Expenses

General and administrative expenses were $56.3 million for the six months ended June 30, 2022 compared to $13.7 million for the six months ended June 30, 2021. The increase of $42.7 million was primarily due to increases of $25.1 million in payroll and personnel-related costs, including stock-based compensation, $7.5 million in consulting expense, $4.4 million in professional services and $2.4 million in insurance expense. The increase in payroll and personnel-related costs was primarily due to hiring additional personnel in commercial and general and administrative functions to support our growth, as well as commercialization and launch preparation initiatives. The increases in professional services, consulting expenses and insurance expenses were primarily due to an increase in spending for commercial readiness activities and operations as a public company.

Other Income (Expense), Net

Interest Income

Interest income for the six months ended June 30, 2022 was $0.5 million compared to less than $0.1 million for the six months ended June 30, 2021. The increase was primarily attributable to higher investment balances driven by our proceeds received from our January 2022 initial public offering, resulting in higher interest earned.

Change in Fair Value of Convertible Notes

The change in fair value of convertible notes was zero for the six months ended June 30, 2022 due to conversion to preferred stock in July 2021, compared to $5.2 million for the six months ended June 30, 2021. The $5.2 million recorded for the six months ended June 30, 2021 represented a loss in fair value related to our 2021 Notes.

Other (Expense) Income, Net

Other expense, net was $0.1 million for the six months ended June 30, 2022, compared to other income of $0.2 million for the six months ended June 30, 2021. The $0.2 million of other income in the six months ended June 30, 2021 was primarily related to the forgiveness of the PPP loan.

Income Taxes

Provision for income taxes was $0.3 million for the six months ended June 30, 2022 compared to zero for the six months ended June 30, 2021, and consists of current income tax expense arising from activities of our foreign subsidiaries.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have incurred significant operating losses and generated revenues through five grants from the Grantors. To date, we have financed our operations primarily through the sale and issuance of common stock, convertible preferred stock, convertible notes, grant agreements with the Grantors and, to a lesser extent, a government loan. As of June 30, 2022, we had cash, cash equivalents and short-term investments of $206.7 million.

From inception through June 30, 2022, we have raised $431.2 million in aggregate proceeds, net of issuance costs, primarily from the issuance of common stock, convertible preferred stock, convertible notes and grant agreements. In July 2021, we issued and sold shares of Series C-1 preferred stock for an aggregate purchase price of approximately $135.0 million. The 2021 Notes automatically converted into shares of Series C-2 preferred stock pursuant to their original terms in July 2021 in connection with our sale of Series C-1 preferred stock. On January 11, 2022, we completed our initial public offering, pursuant to which we received

aggregate net proceeds of $196.4 million, including the partial exercise by the underwriters of their option to purchase additional shares, and after deducting underwriting discounts and commissions and other offering costs. Based on our current operational plans and assumptions, we believe that our existing cash, cash equivalents and short-term investments, together with the net proceeds from our initial public offering, 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.

Capital Resources

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities, manufacturing and clinical trials of AMX0035 and any future product candidates, implement our commercialization plans for ALBRIOZA in Canada, and prepare for the commercial launch of AMX0035 in other jurisdictions, if approved. In addition, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Our expenses will also increase as we:

- continue our research and development efforts, including our ongoing global Phase 3 PHOENIX trial of AMX0035 for the treatment of ALS;
- continue to commercialize AMX0035 (also known as ALBRIOZA in Canada) for the treatment of ALS in Canada, and pursue launch of AMX0035 in the United States and Europe, if approved;
- pursue investigational new drug applications ("INDs") of AMX0035 for the treatment of Wolfram syndrome and potentially for other indications;
- conduct preclinical studies and clinical trials 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 marketing approval for AMX0035 or any future product candidates and to support manufacturing on a commercial scale;
- seek regulatory approvals for AMX0035 or any future product candidates that successfully complete clinical trials, if any;
- hire and retain additional personnel, such as non-clinical, clinical, quality assurance, regulatory affairs, manufacturing, distribution, legal, compliance, finance, general and administrative, commercial and scientific personnel;
- develop, maintain, expand and protect our intellectual property portfolio; and
- continue to transition our organization to being a public company.

We are a publicly traded company and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002, as well as rules adopted by the SEC and the Nasdaq Global Select Market, require public companies to implement specified corporate governance practices that are currently not applicable to us as a private company. Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002 ("Section 404") we will first be required to furnish a report by our management on our internal control over financial reporting for the year ending December 31, 2022. However, while we remain an emerging growth company and/or a smaller reporting company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. We expect these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

Based on our current operational plans and assumptions, we expect that our current cash, cash equivalents and short-term investments, combined with the net proceeds from our initial public offering will be sufficient to fund operations for at least twelve months after the date of 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. As we progress with our development activities and the regulatory review process, we expect to incur significant commercialization expenses related to product manufacturing, pre-commercial activities and commercialization.

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 and any future product candidates;
- the costs, timing and outcome of commercialization activities, including manufacturing, marketing, sales and distribution for ALBRIOZA in Canada and for AMX0035, if approved, in other territories or for any future product candidates for which we receive marketing approval;
- the costs, timing and outcome of regulatory review of AMX0035 and any future product candidates;
- our ability to establish and maintain collaborations 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 and any future product candidates, including as result of the ongoing COVID-19 pandemic or other pandemics or disruptions;
- the costs of expanding our facilities to accommodate our expected 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 AMX0035 and any future product candidates, if and when approved; and
- the costs of operating as a public company.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to 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 Six Months Ended June 30, 2022 and 2021

The following table summarizes our sources and uses of cash for the periods presented:

	Six months ended June 30,			
	2022	2021	$ Change	% Change
	(in thousands)			
Net cash used in operating activities	$ (87,096)	$ (25,690)	$ (61,406)	239%
Net cash used in investing activities	(94,849)	(14)	(94,835)	677,393%
Net cash provided by financing activities	199,627	27,920	171,707	615%
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(203)	2	(205)	(10,250)%
Net increase in cash, cash equivalents and restricted cash	$ 17,479	$ 2,218	$ 15,261	688%

Operating Activities

During the six months ended June 30, 2022, operating activities used $87.1 million of cash, primarily resulting from our net loss of $101.9 million, offset by $10.1 million of non-cash stock-based compensation expense, $0.1 million of depreciation expense, $0.1 million net amortization of premiums and discounts on investments, and $4.7 million of net cash provided by changes in our operating assets and liabilities.

Net cash used in our operating assets and liabilities primarily consisted of a $4.1 million increase in prepaid expenses and other current assets due to recognition of deferred offering costs related to the initial public offering, and an increase of $0.5 million in other assets, offset by a $3.8 million increase in accounts payable, a $4.8 million increase in accrued expenses and deferred rent due to increased spending for external research and development to support our growth, and $0.8 million decrease in operating lease right-of use assets.

During the six months ended June 30, 2021, operating activities used $25.7 million of cash, primarily resulting from our net loss of $36.4 million, $4.3 million of net cash provided by changes in our operating assets and liabilities, offset by $5.2 million of change in fair value of convertible notes and $1.2 million of non-cash stock compensation expense.

Net cash provided by changes in our operating assets and liabilities primarily consisted of a $0.9 million decrease in accounts payable, $0.1 million decrease in other current assets, offset by $5.7 million increase in accrued expenses and deferred rent and a $0.7 million increase in prepaid expenses and other current assets.

Investing Activities

During the six months ended June 30, 2022, net cash used in investing activities was $94.8 million, resulting from $1.4 million of purchases of property and equipment and $154.3 million of purchases of short-term investments, offset by $60.9 million of investments that matured during the period.

During the six months ended June 30, 2021, net cash used in investing activities was less than $0.1 million, driven by purchases of property and equipment.

Financing Activities

During the six months ended June 30, 2022, net cash provided by financing activities was $199.6 million. This amount consisted of $216.0 million of gross proceeds from sale of common stock from our initial public offering, offset by $15.1 million of underwriters' discounts and $1.5 million of offering costs paid during the period. Offering costs remaining to be paid as of June 30, 2022 totaled $0.5 million, coupled with offering costs paid in prior periods, results in total net proceeds from the initial public offering of $196.4 million.

During the six months ended June 30, 2021, net cash provided by financing was $27.9 million. This amount consisted of $14.3 million of proceeds from the issuance of convertible notes to related parties, $11.9 million of proceeds from the issuance of the convertible notes, $2.1 million of proceeds received in advance of issuance of our Series C-1 redeemable convertible preferred stock, and less than $0.1 million of proceeds from exercises of stock options, offset by a $0.3 million forgiveness of PPP loan.

In April 2020, we received the PPP Loan from First Republic Bank. Under the terms of the CARES Act and the PPP Loan, all or portion of the principal amount of the PPP Loan was subject to forgiveness so long as, over the 24-week period following our receipt of the proceeds of the PPP Loan, we used those proceeds for payroll costs, rent, utility costs or the maintenance of employee and compensation levels. The PPP Loan was forgiven in March 2021 and notwithstanding the forgiveness of the PPP Loan, we repaid it in full on October 7, 2021.

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

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 most recent Annual Report on Form 10-K with the exception of significant accounting policies related to the adoption of FASB ASC Topic 842, Leases, effective January 1, 2022. Refer to Note 5 to the consolidated financial statements contained in this Quarterly Report for a discussion of FASB ASC Topic 842, *Leases*.

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our 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 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.

Emerging Growth Company and Smaller Reporting Company Status

The Jumpstart Our Business Startups Act of 2012 ("JOBS Act") permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, we will not be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies, and our condensed consolidated financial statements may not be comparable to other public companies that comply with new or revised accounting pronouncements as of public company effective dates. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

We will cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.07 billion or more, (ii) the last day of our fiscal year following the fifth anniversary of the date of the closing of our initial public offering, (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years or (iv) the date on which we are deemed to be a large, accelerated filer under the rules of the Securities and Exchange Commission.

We are also a “smaller reporting company”, and we will continue to be a smaller reporting company until the first quarter of the fiscal year following the determination that the market value of our stock held by non-affiliates is more than $250 million measured on the last business day of our second fiscal quarter, or our annual revenue are more than $100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth 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 the two most recent fiscal years of audited financial statements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company as defined by Rule 12b-2 of the Exchange Act and are not required to provide the information required under this item.

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 Chief Executive Officers and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2022. Based upon the evaluation, our Chief Executive Officers and Chief 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 six months ended June 30, 2022 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.

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have an adverse effect on our business, operating results or financial condition.

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 incurred significant losses since our inception. Until we are able obtain and maintain regulatory approvals for our product candidate, we anticipate that we will continue to incur significant losses for the foreseeable future.
- We have only recently launched ALBRIOZA in Canada and prior to its launch have never generated revenue from product sales. Even if our launch of ALBRIOZA in Canada is successful, unless AMX0035 is approved in other jurisdictions, we may never be profitable.
- We have a limited operating history and our only product candidate, AMX0035 (known as ALBRIOZA in Canada), has received marketing authorization (with conditions) only in Canada with limited commercial sales to date, which may make it difficult to evaluate the prospects for our future viability.

Risks Related to Commercialization of AMX0035 or Future Product Candidates

- We currently have limited sales and marketing capabilities. If we are unable to establish effective sales and marketing capabilities or enter into agreements with third parties to market and sell AMX0035 and any future product candidates that may be approved, we may not be successful in commercializing AMX0035 and any future product candidates if and when approved, and we may be unable to generate any product revenue.
- In Canada, and even if AMX0035 or any future product candidate of ours receives regulatory approval in other jurisdictions, AMX0035 (known as ALBRIOZA in Canada) may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.
- Healthcare insurance coverage and reimbursement, both from public drug plans and private health care insurers, may be limited or unavailable for ALBRIOZA in Canada, and for AMX0035 and any future product candidates, if approved, which could make it difficult for us to sell any product candidates or therapies profitably.

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

- We currently depend on the success of AMX0035, which recently received marketing authorization with conditions from Health Canada for the treatment of ALS and has a pending NDA before the FDA and a MAA before the EMA. If we are unable to obtain and maintain regulatory approval for, and successfully commercialize, AMX0035, or experience significant delays in doing so, our business may be materially harmed.
- The delay or denial of regulatory approval, inability to maintain regulatory approval, or the requirement to resubmit any marketing application with additional data or information for AMX0035 in any jurisdiction could mean that we need to delay or even cease operations, and a delay in obtaining or inability to maintain such approval would delay commercialization of AMX0035 and adversely impact our ability to generate revenue, our business and our results of operations.

- Although we have received marketing authorization with conditions of AMX0035 (also known as ALBRIOZA) in Canada, we have limited experience commercializing a product and may experience delays or unexpected difficulties in obtaining and maintaining regulatory approvals for AMX0035 for our initial or potential additional indications.
- AMX0035 is a fixed-dose combination drug product and certain regulatory authorities, including the FDA, 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 concentrated our research and development efforts on the treatment of neurodegenerative and central nervous system ("CNS") disorders, a field that has seen very limited success in product development.
- The regulatory approval processes of the FDA, Health Canada, the EMA and other comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain or maintain regulatory approval for AMX0035 or any future product candidates, our business will be substantially harmed.
- Competitive products may reduce or eliminate the commercial opportunity for AMX0035 for our current or future indications. 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 may be adversely affected.

Risks Related to Our Dependence on Third Parties

- We may in the future enter into collaborations with third parties for the development and commercialization of AMX0035 or any future product candidates, and our prospects with respect to those 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 or maintain regulatory approval or successfully commercialize AMX0035 or any future product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.
- Our use of third parties to manufacture AMX0035 may increase the risk that we will not have sufficient quantities of AMX0035, products, or necessary quantities of such materials on time or at an acceptable cost.

Risks Related to Our Intellectual Property

- Our commercial success depends on our ability to protect our intellectual property and proprietary technology, and to achieve data and market exclusivities in applicable markets.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

- A pandemic, epidemic, or outbreak of an infectious disease, such as the ongoing and evolving COVID-19 pandemic, 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 depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.
- We expect to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

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.
- 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.
- We previously identified material weaknesses in our internal control over financial reporting. If we fail to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations, meet our reporting obligations or prevent fraud, and investor confidence in our company and the market price of our common stock may be materially and adversely affected.

Risks Related to Our Financial Position and Need for Capital

We have incurred significant losses since our inception. Until we are able to obtain and maintain regulatory approvals for our product candidate, we anticipate that we will continue to incur 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. Although we have invested substantial resources into our product development efforts and toward the commercialization of AMX0035, which has received marketing authorization with conditions from Health Canada, we have not yet received approval in any other jurisdiction and have not generated any significant revenue from product sales to date in Canada or elsewhere. We will continue to incur significant research and development and other expenses related to clinical development, preparation for commercialization, and ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception. 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, and preparation for commercialization. Our financial condition and operating results, including our expenses and net losses, may fluctuate significantly from quarter to quarter and year to year. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance. Additionally, net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. Our net losses were $54.1 million and $101.9 million for the three and six months ended June 30, 2022, respectively, and $21.9 million and $36.4 million for the three and six months ended June 30, 2021, respectively. As of June 30, 2022, we had an accumulated deficit of $257.8 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek to obtain and maintain regulatory approvals for our product candidate, AMX0035, for the treatment of ALS, AD and potential additional indications, as well as for future product candidates we may develop.

We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct clinical trials for AMX0035 for the treatment of ALS, AD and potential additional indications;
- initiate and continue research, preclinical and clinical development efforts for any future product candidates;
- seek to identify additional product candidates;
- seek to obtain and maintain regulatory approvals in Canada, the United States, the EU, and other geographies for AMX0035 for the treatment of ALS, AD and other indications that successfully complete clinical development;
- experience any delays or encounter any issues with any of the above, including but not limited to completion of post-marketing requirements, the potential that the FDA or other regulators require additional data to support the approval of AMX0035 for ALS, failed studies, negative or mixed clinical trial results, safety issues or other regulatory challenges, the risk of which in each case may be exacerbated by the ongoing COVID-19 pandemic;
- establish sales, marketing, distribution, manufacturing, supply chain and other commercial infrastructure to commercialize various products for which we have and may in the future obtain regulatory approval;
- add operational, financial and management information systems and personnel, including personnel to support our product candidate development and help us comply with our obligations as a public company;
- hire and retain additional personnel, such as clinical, quality control, scientific, commercial and administrative personnel;
- maintain, expand and protect our intellectual property portfolio;
- add equipment and physical infrastructure to support our research and development; and
- acquire or in-license other product candidates and technologies.

We are currently building our infrastructure, including sales and marketing and production capabilities, for commercialization in Canada, and in the United States in anticipation of potential regulatory approval in the near term. As of June 30, 2022, we had 226 full-time employees.

Our expenses could increase beyond our expectations if we are required by Health Canada, the FDA, the EMA, or other regulatory authorities to perform clinical trials or conduct other studies in addition to those that we currently expect, or if there are any delays in establishing appropriate manufacturing arrangements for or in completing our clinical trials or the development of AMX0035 or any future product candidates we may develop.

We have only recently launched ALBRIOZA in Canada and prior to its launch have never generated revenue from product sales. Even if our launch of ALBRIOZA in Canada is successful, unless AMX0035 is approved in other jurisdictions, we may never be profitable.

Our ability to become and remain profitable depends on our ability to generate revenue. Other than ALBRIOZA in Canada, we have no approved products for commercial sale and have not yet generated any significant revenue from product sales. Successful commercialization will require achievement of many key milestones, which vary by jurisdiction and may include demonstrating safety and efficacy in clinical trials, obtaining and maintaining 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. Even if we successfully develop and commercialize ALBRIOZA in Canada, unless AMX0035 is approved in other jurisdictions, we may be unable to achieve or maintain profitability. 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 become and remain profitable may depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. If we continue to suffer losses as we have in the past, investors may not receive any return on their investment and may lose their entire investment.

We have a limited operating history and our only product candidate, AMX0035 (known as ALBRIOZA in Canada), has received marketing authorization (with conditions) only in Canada with limited commercial sales to date, which may make it difficult to evaluate the prospects for our future viability.

We are a biopharmaceutical company founded in 2013, and our operations to date have been limited to organizing, staffing and financing our company, raising capital, conducting research and development activities, including preclinical studies and clinical trials and preparing for commercialization of AMX0035. We have not yet demonstrated an ability to generate significant revenues, or clearly conduct sales and marketing activities necessary for successful product commercialization. In July 2022, AMX0035 received marketing authorization with conditions from by Health Canada for the treatment of ALS, and we have a pending NDA before the FDA and a pending MAA before the EMA. Accordingly, you should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in the early commercial stage, especially 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 with these activities.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We are transitioning from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We will require substantial additional funding to meet our financial needs and to pursue our business objectives. If we are unable to raise capital 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 continue to spend substantial amounts to continue the clinical development of AMX0035 and the preclinical and clinical development of any future product candidates. If we are able to obtain and maintain marketing approval for AMX0035 or any future product candidates that we develop, including any indication for which we are developing or may develop AMX0035, we will require significant additional amounts of cash in order to launch and commercialize such product candidates to the extent that such launch and commercialization are not the responsibility of a collaborator that we may contract with in the future. In addition, other unanticipated costs may arise in the course of our development and commercialization 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 the treatment of ALS, AD and potential additional indications, as well as any future product candidates we may develop;
- the timing of, and the costs involved in, obtaining and maintaining marketing approvals for AMX0035 for the treatment of ALS, AD and potential additional indications, and any future product candidates we may develop and pursue;

- the number of future product candidates that we may pursue and their development requirements;
- the costs of commercialization activities for AMX0035 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 for any approved indications or any future product candidates;
- the extent to which we in-license or acquire rights to other products, product candidates or technologies;
- our headcount growth and associated costs as we expand our research and development, increase our office space, and establish a commercial infrastructure;
- 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 ongoing COVID-19 pandemic and actions taken to slow its spread, global economic instability and geopolitical events, including the conflict in Ukraine, the global credit and financial markets have experienced extreme volatility and disruptions, including diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment 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 in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of AMX0035 or any future product candidates or other research and development initiatives. We may need to seek collaborators for AMX0035 and any 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 and any 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 the net proceeds from our initial public offering, together with our existing cash, cash equivalents, and short-term investments, will be sufficient to meet our anticipated operating and capital expenditure requirements for at least twelve months after the date of this filing. 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 increase in connection with our planned operations. Unless and until we can generate a substantial amount of revenue from AMX0035 or any future product candidates, we expect 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. In addition, securing financing could 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 and commercialization of AMX0035 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, and local 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.

Risks Related to Commercialization of AMX0035 or Future Product Candidates

We currently have limited sales and marketing capabilities. If we are unable to establish effective sales and marketing capabilities or enter into agreements with third parties to market and sell AMX0035 and any future product candidates that may be approved, we may not be successful in commercializing AMX0035 and any future product candidates if and when approved, and we may be unable to generate any product revenue.

We recently launched ALBRIOZA in Canada, and if approved, we also intend to seek to commercialize AMX0035 in the United States and the EU directly with specialized teams, given the relative rarity of certain of the indications we are targeting. We are currently continuing to build the marketing and sales team for the marketing, sales and distribution of AMX0035 and any future product candidates, if approved. In order to commercialize AMX0035 for the treatment of ALS, AD and other indications, or any of our future product candidates that may be approved, we must build, on a territory-by-territory basis, marketing, sales, distribution, managerial and other capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, our current efforts toward recruiting and training a commercial organization are expensive and time consuming and could delay any further product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. If we fail to obtain or maintain approval or if for any reason a potential commercial launch is otherwise delayed, we would not realize the benefits of any pre-commercial activity expenditures made to date. This may be costly and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize AMX0035 or any future product candidates, if approved, on our own include:

- the inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians to prescribe AMX0035 or any future product that we may develop;
- any views or opinions expressed by ALS or AD community organizations about the efficacy of AMX0035;
- the lack of complementary treatments to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- the availability of adequate coverage by and reimbursement from government and third-party payors; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or profitability from these revenue streams is likely to be lower than if we were to market and sell any product candidates that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market AMX0035 or any of our future product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market AMX0035 or any future product candidates effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we may not be successful in commercializing AMX0035 or any future product candidates.

Our efforts to educate the ALS, AD and other neurodegenerative disease medical communities and payors on the benefits of AMX0035 or any future product candidates may require significant resources given the relative rarity of certain of the indications we are targeting, and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of AMX0035 or any future product candidates, and the indications we are targeting. Even if AMX0035 or any future

product candidates are approved in any jurisdiction, if we are unable to successfully market our products successfully, we will not be able to generate significant revenues from such products.

If we are unable to expand our marketing and distribution capabilities or enter into agreements with third parties to market and sell any of AMX0035 or future product candidates for which we obtain marketing approval, we will be unable to generate any product revenue.

To successfully commercialize any products that may result from our development activities, we need to continue to expand our marketing and distribution capabilities, either on our own or with others. The development of our own marketing and distribution effort is, and will continue to be, expensive and time-consuming and could delay any further product launch. Moreover, we cannot be certain that we will be able to develop this capability successfully. 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 or any 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 also face competition in our search for third parties to assist us with the sales and marketing efforts of AMX0035 and any 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.

The market for AMX0035 for ALS, AD and other neurodegenerative diseases and for any future product candidates we may develop may be smaller than we expect.

We focus our research and product development on treatments of neurodegenerative diseases. 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 or any 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 ultra-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. For example, as we begin to commercialize ALBRIOZA in Canada and AMX0035, if approved, in other jurisdictions, and learn more about market dynamics and engage with regulators on additional potential marketing approvals, our view of our products' initial potential market opportunity will become more refined. For example, we are now initially focused primarily on the annual incidence of ALS. This means the initial market opportunity for AMX0035 and any future product candidates may be smaller than the total addressable market opportunity that could be achieved over time. If we are unable to successfully commercialize AMX0035 or any 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, 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 Canada, the United States, the EU 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.

In Canada, and even if AMX0035 or any future product candidate of ours receives regulatory approval in other jurisdictions, AMX0035 (known as ALBRIOZA in Canada) may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.

Even if AMX0035 for the treatment of any indication, or any 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 take their patients off their current medications and switch their treatment regimen to AMX0035. Further, patients often acclimate to the treatment regime they are currently taking and do not want to switch unless their physicians recommend switching products or are required to switch due to lack of coverage and adequate reimbursement. In addition, even if we are able to demonstrate our product candidates' safety and efficacy to Health Canada, the FDA, the EMA 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 or any 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 become profitable. The degree of market acceptance of AMX0035 and any future product candidates, once 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;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product's convenience 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 or any other potential product candidate of ours that obtains regulatory approval to achieve market acceptance or commercial success would adversely affect our business prospects.

Off-label use for the treatment of ALS of PB which is available as a generic drug, along with the potential sale in some jurisdictions of TURSO, which preparations are of unknown identity and may not be legally sold for the treatment of ALS, expose us to additional risks that could reduce or eliminate the commercial opportunity for AMX0035.

We are developing AMX0035 as 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.

TURSO is being marketed in preparations of unknown identity and without approval for the treatment of ALS in some jurisdictions, including the United States. We face the risk that healthcare professionals may prescribe PB for the treatment of ALS and recommend that patients obtain a commercial preparation of TURSO not labeled or marketed for the treatment of ALS on the belief that this combination could replicate the benefits of AMX0035. Patient-directed treatment with TURSO for ALS may also arise in certain jurisdictions if the Phase 3 clinical trial to assess the safety and efficacy of TURSO in patients with ALS conducted by Humanitas Mirasole SpA ("Humanitas") in the EU reports positive results. While these practices are not recommended by the medical community and have not been approved by any regulatory authority, they may nonetheless impact our sales of ALBRIOZA in Canada and AMX0035, if approved in other jurisdictions, and/or public perception of AMX0035 in the United States or abroad.

If the FDA, Health Canada, the EMA or other comparable foreign regulatory authorities approve generic versions of AMX0035 or any future product candidate of ours that receives regulatory approval, or such authorities do not grant our products appropriate periods of non-patent exclusivity before approving generic versions of such products, the sales of such products could be adversely affected.

In the United States, once an NDA is approved, the product covered thereby becomes a "reference listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," or the Orange Book. Manufacturers may seek approval of generic versions of reference listed drugs through submission of abbreviated new drug applications ("ANDAs") in the United States. In support of an ANDA, a generic manufacturer generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration, and adequate labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning, in part, that it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Moreover, many states allow or require substitution of therapeutically equivalent generic drugs at the pharmacy level even if the branded drug is prescribed. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug may be lost to the generic product.

The FDA may not finally approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity ("NCE"). For the purposes of this provision, an NCE is a drug that contains no active moiety that has previously been approved by the FDA in any other NDA. An active moiety is the molecule or ion responsible for the physiological or pharmacological action of the drug substance. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the listed drug is invalid, unenforceable or will not be infringed by the generic product. In that case, the applicant may submit its application four years following approval of the listed drug and seek to launch its generic product even if we still have patent protection for our product unless an infringement suit is timely filed by the NDA or patent holder in which case the FDA cannot approve the ANDA for 30 months unless a court decision in favor of the generic manufacturer is issued earlier. For fixed dose combination products, the FDA has taken the position that a combination product will be eligible for NCE exclusivity (also known as data exclusivity) if it contains a new active moiety, even if the fixed-combination also contains a drug substance with a previously approved active moiety.

The FDA may determine, however, that AMX0035 is not eligible for NCE exclusivity, if and when FDA approves an NDA for the product. For example, even under its fixed dose combination product policies, the FDA may find that the active moieties in AMX0035 have been previously approved and, therefore, NCE exclusivity is not available for AMX0035. The regulatory authorities in Canada and the EU may reach the same conclusions as the FDA since the determination of data exclusivity for new drug products in those jurisdictions is very similar to that of the United States.

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 essential to the approval of the NDA. This form of data exclusivity is known as New Clinical Investigation ("NCI") exclusivity. If AMX0035 is approved with only NCI exclusivity, generic manufacturers may file their ANDAs anytime following approval of AMX0035 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 United States 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 United States of such drug. If AMX0035 is granted orphan drug exclusivity, the FDA cannot finally approve a generic or a brand product that contains the same active moiety for the same orphan indication as AMX0035, for a period of seven years, subject to certain exceptions.

In Canada, ursodoxicoltaurine (one of the active pharmaceutical ingredients in AMX0035) is designated as an innovative drug under Section C.08.004.1 of the *Food and Drug Regulations*, which entitles the drug to an eight-year period of market exclusivity. A generic drug manufacturer seeking marketing authorization on the basis of a direct or indirect comparison to AMX0035 is prevented from filing its drug submission for the first six years of the eight-year period.

After six years, a generic manufacturer may file its drug submission with Health Canada, but the Notice of Compliance (NOC or marketing approval) for the generic product cannot be granted by Health Canada for eight years from the date that AMX0035 was granted its NOC. There is no regulatory provision in Canada that provides orphan drug exclusivity to approved products for rare diseases.

In the EU, innovative medicinal products (including both small molecules and biological medicinal products), sometimes referred to as new active substances ("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 marketing 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. Amylyx has applied for NAS

status for AMX0035 in the EU. Irrespective of the NAS status, we expect that AMX0035 will be eligible for Regulatory Data Protection and/or Orphan Market Exclusivity if the orphan designation is maintained.

Competition that AMX0035 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.

AMX0035 or any future product candidates for which we, or any future collaborators, obtain regulatory approval will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. If approved, AMX0035 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 or any 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, Health Canada, the EMA 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 for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or other products that are otherwise unfit for distribution in the United States. 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 United States to implement a Risk Evaluation and Mitigation Strategy.

The FDA, Health Canada, the EMA 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. For example, 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 or any of our 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, and may also impose limitations on our promotional activities with health care professionals.

Post-marketing requirements in Canada are similar to those in the United States. Following the approval of our NDS with conditions, Health Canada requires that we submit a Risk Management Plan ("RMP"). Health Canada may, as part of the RMP, require that we conduct additional clinical studies. For example, one of the conditions of the marketing authorization in Canada of ALBRIOZA is the provision of data from our ongoing PHOENIX trial and other additional planned or ongoing studies. Standard pharmacovigilance activities are also required for any marketing drug product. Any labelling changes or changes in the product supply chain would require a submission to Health Canada for approval before the change may be implemented. Our advertising may be scrutinized by competitors or by HCPs, and complaints could be made to Health Canada or other agencies. Reimbursement in Canada is complex and will require submissions to both public and private payors to gain access to prescription drug formulary lists. In addition, if there are any patents associated with AMX0035, the product will be subject to price regulation by the Patented Medicine Prices Review Board ("PMPRB").

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.

Healthcare insurance coverage and reimbursement, both from public drug plans and private health care insurers, may be limited or unavailable for ALBIOZA in Canada, and for AMX0035 and any future product candidates, if approved, which could make it difficult for us to sell any product candidates or therapies profitably.

The success of AMX0035 and any future product candidates, if approved, depends on the availability of adequate coverage and reimbursement from third-party payors. Because AMX0035 and any 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 and any 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 or any 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2021.

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 United States, 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. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services ("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 United States 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 United States, 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 and any future product candidates we may develop, if approved. In many countries, particularly the countries of the EU, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. For example, in Canada, price negotiations with provincial authorities can take more than 18 months before there are agreed-upon pricing and reimbursement rates. Prior to these negotiations, a review by agencies known as the Canadian Agency for Drugs and Technologies in Health, and l'Institut national d'excellence en santé et en services sociaux are conducted to assess the value that a medicine will provide to the health system. For patented medicines, the PMPRB has jurisdiction over the price at which the medicine is sold, and PMPRB's assessment of an acceptable price can impact negotiations with payors. Such negotiations may also result in additional studies and rationale required for combination products before reimbursement will be granted. As a result, 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 United States. 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 United States, the reimbursement for AMX0035 and any future product candidates we may develop may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of product candidates. Patients are unlikely to use AMX0035 or any future product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of AMX0035 or any future product candidates. Because AMX0035 and any future product candidates may have a higher cost of goods than conventional therapies and may require long-term follow-up evaluations, the risk that coverage and reimbursement rates may be inadequate for us to achieve profitability may be greater. While we have received a positive response from some providers in Canada following Health Canada's approval of AMX0035, there is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for AMX0035 and any future product candidates.

Moreover, increasing efforts by governmental and other third-party payors in Canada, the EU, the United States and other foreign jurisdictions 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 AMX0035 or any future product candidates. There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. We expect to experience pricing pressures in connection with the sale of AMX0035 or any future product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, cost containment initiatives and additional legislative changes.

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 Annual Report on Form 10-K for the fiscal year ended December 31, 2021.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs.

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 or any future product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used. 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, attain profitability or commercialize our product candidates.

Moreover, increasing efforts by governmental and third-party payors in the United States 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 United States 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.

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 United States 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. The EU provides options for the EU Member States to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for drug products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. 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 study or other studies that compare the cost-effectiveness of AMX0035 or any 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 or any future product candidates in those countries would be negatively affected.

Our relationships with healthcare providers and physicians 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 United States 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 ("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 Annual Report on Form 10-K for the fiscal year ended December 31, 2021.

The distribution of pharmaceutical products is 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.

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 EU, including personal health data, is subject to the EU General Data Protection Regulation ("the GDPR") which took effect across all member states of the European Economic Area ("EEA") in May 2018. 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 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 increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the United States, 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 United States. 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 Euros, 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. In addition, the GDPR provides that EU member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Similar actions are either in place or underway in the United States. 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—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 (the "Common Rule"). Many other states are considering similar legislation. A broad range of legislative measures also has been introduced at the federal level. Accordingly, failure to comply with federal and state laws (both those currently in effect and future legislation) regarding the privacy and security of personal information could expose us to fines and penalties under such laws. There also is the threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with these requirements is rigorous and time-intensive and requires significant resources and a 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 collected in the EU. 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.

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

We currently depend on the success of AMX0035, which recently received marketing authorization with conditions from Health Canada for the treatment of ALS and has a pending NDA before the FDA and a MAA before the EMA. If we are unable to obtain and maintain regulatory approval for, and successfully commercialize, AMX0035, or experience significant delays in doing so, our business may be materially harmed.

We currently only have one product candidate, AMX0035, and our business and future success depends entirely on our ability to develop, obtain and maintain regulatory approvals for, and then successfully commercialize, AMX0035, which we are developing for patients with ALS and AD. To date, we have obtained limited clinical trial data supporting AMX0035, having only completed a clinical trial of 137 patients with ALS and a clinical trial in 95 patients with AD. We are conducting a global Phase 3 clinical trial of AMX0035 in ALS and intend to conduct additional clinical trials for other indications in the future. This may make an investment in our company riskier than similar companies that have multiple product candidates in active development that may be able to better sustain failure of a lead product candidate.

We recently received marketing authorization with conditions from Health Canada for AMX0035 for the treatment of ALS and have a pending NDA before the FDA and a MAA pending before the EMA. Accordingly, we are investing the majority of our efforts and financial resources in the further development and commercialization of our product candidate, AMX0035, for the treatment of ALS, AD and other diseases. Successful continued development and additional regulatory approvals of AMX0035 for our initial and potential additional indications is critical to the future success of our business. We will need to raise sufficient funds for, and successfully enroll and complete, our clinical development of AMX0035 for the treatment of ALS, AD and other indications. The future regulatory and commercial success of AMX0035 or any 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 or any future product candidates in the intended populations;
- satisfaction of applicable regulatory requirements, including to satisfy applicable rules governing fixed dose combination products;
- 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 new chemical entity and new clinical investigation data exclusivity and orphan drug market exclusivity;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for AMX0035 or any future product candidates;
- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of AMX0035 or any future product candidates;
- entry into collaborations to further the development of AMX0035 or any future product candidates;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, including of ALBRIOZA in Canada and AMX0035, if and when approved in other jurisdictions, whether alone or in collaboration with others;
- successfully launching commercial sales of ALBRIOZA in Canada and AMX0035 or any future product candidates, if and when approved in other jurisdictions;
- acceptance of AMX0035 or any other products, if and when approved, by patients, the medical community and third-party payors;
- obtaining and maintaining third-party coverage and adequate reimbursement;

- maintaining a continued acceptable safety profile of the products following approval;
- effectively competing with other 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, receive regulatory approval for, or successfully commercialize AMX0035 for the indications we are developing it for, 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 a NDS to Health Canada, a NDA to the FDA or an MAA to the EMA, and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval for AMX0035 for any indication, any such approval may be subject to limitations on the indications or uses or the patient populations for which we may market the product. 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 AMX0035 for any indication in any jurisdiction. If we or any of our future collaborators are unable to develop, or obtain and maintain regulatory approval for, or, if approved, successfully commercialize AMX0035 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 AMX0035, or to satisfy other regulatory requirements could adversely affect our development efforts for AMX0035 in other indications.

The delay or denial of regulatory approval, inability to maintain regulatory approval, or the requirement to resubmit any marketing application with additional data or information for AMX0035 in any jurisdiction could mean that we need to delay or even cease operations, and a delay in obtaining or inability to maintain such approval would delay commercialization of AMX0035 and adversely impact our ability to generate revenue, our business and our results of operations.

If we are not successful in commercializing AMX0035, or are significantly delayed in doing so, our business will be materially harmed, and we may need to curtail or cease operations. The research, testing, manufacturing, labeling, approval, sale, marketing and distribution of drug products are subject to extensive regulation by Health Canada, the FDA, the EMA, and other regulatory agencies in the United States and other countries, and such regulations differ from country to country. We are not permitted to market AMX0035 until we receive approval or marketing authorization from the relevant regulatory authority. We recently received marketing authorization with conditions from Health Canada for AMX0035 (ALBRIOZA) for the treatment of ALS. One of the conditions of the approval is the provision of data from our ongoing PHOENIX trial and additional planned or ongoing studies. We also have a pending NDA before the FDA and a MAA before the EMA. Health Canada, the FDA, the EMA or any other foreign regulatory agency can delay, limit, deny or withdraw approval to market AMX0035 for many reasons, including:

- our inability to demonstrate to the satisfaction of Health Canada, the FDA, the EMA or any other applicable foreign regulatory agency that AMX0035 is safe and effective for the requested indication;
- our inability to gain agreement from applicable foreign regulatory authorities that AMX0035 is appropriate for approval under applicable regulatory pathways;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's disagreement with the interpretation of data from nonclinical and clinical studies and trials, such as the FDA's differing interpretations of certain data from our CENTAUR trial and OLE trial as presented at the meeting of the FDA's Advisory Committee on March 30, 2022;
- our inability to demonstrate that the clinical and other benefits of AMX0035 outweigh any safety or other perceived risks;
- our ability to enroll an adequate number of patients in and successfully complete our ongoing global Phase 3 PHOENIX trial;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's requirement for additional nonclinical or clinical studies or trials, including studies to satisfy applicable rules governing fixed dose combination products or post-market requirements;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's having differing requirements for the trial protocols used in our clinical trials;
- Health Canada's, the FDA's, the EMA's or any other applicable foreign regulatory agency's non-approval of the formulation, labeling and/or the specifications of AMX0035;

- Health Canada's, the FDA's, the EMA'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 of Health Canada, the FDA, the EMA 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 Health Canada, the FDA, the EMA or other regulatory approval processes and are commercialized.

Even if we eventually complete clinical testing and receive approval of an NDS, NDA, MAA or other foreign marketing authorization for AMX0035, the FDA or the applicable foreign regulatory agency may grant approval contingent on the performance of costly additional clinical trials, which may be required after approval. The FDA or the applicable foreign regulatory agency may also approve AMX0035 for a more limited indication and/or a narrower patient population than we originally request, and Health Canada, the FDA, the EMA 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. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of AMX0035 and would materially adversely impact our business and prospects.

Although we have received marketing authorization with conditions of AMX0035 (also known as ALBRIOZA) in Canada, we have limited experience commercializing a product and may experience delays or unexpected difficulties in obtaining and maintaining regulatory approval for AMX0035 for our initial or potential additional indications.

We have received marketing authorization with conditions of AMX0035 (ALBRIOZA) only in Canada, and have limited experience commercializing a drug. It is possible that Health Canada, the FDA and the EMA may refuse to accept any or all of our submitted or planned NDSs, NDAs and MAAs for substantive review or may conclude after review of our data that our application is insufficient to obtain regulatory approval for AMX0035 or any future product candidates. For example, the FDA or other regulatory authorities may require completion of our ongoing Phase 3 PHOENIX global clinical trial prior to issuing an approval decision for our marketing applications for AMX0035. If Health Canada, the FDA, and the EMA do not approve any of our submitted or planned NDSs, NDAs or MAAs, such regulatory authorities may require that we conduct additional costly clinical, nonclinical or manufacturing validation studies before they will reconsider our applications or grant full approval. Depending on the extent of these or any other required studies, approval of any NDA or other application that we submit may be significantly delayed, possibly for several years, or may require us to expend more resources than we have available. Any failure or delay in obtaining or maintaining regulatory approvals would prevent or significantly delay us from commercializing AMX0035 for any indication or any other product candidate, generating revenues and achieving and sustaining profitability. It is also possible that additional studies, if performed and completed, may not be considered sufficient by Health Canada, the EMA or FDA to approve any NDS, MAA, NDA or other application that we submit. For example, we recently received marketing authorization with conditions for AMX0035 (ALBRIOZA) from Health Canada. One of the conditions of the marketing authorization is the provision of data from our ongoing PHOENIX trial. There is no guarantee that Health Canada will accept the data from our PHOENIX trial as satisfying the conditions and grant a marketing authorization without conditions for AMX0035 for the treatment of ALS. Health Canada could require Amylyx to make further undertakings with respect to confirmatory clinical trials if it is not satisfied with the data from the PHOENIX trial, in order for ALBRIOZA to continue to be marketed in Canada. If any of these outcomes occur, we may be forced to abandon the development of AMX0035 or any future product candidates, which would materially adversely affect our business and could potentially cause us to cease operations. We face similar risks for our applications in other foreign jurisdictions. In addition, difficulties in obtaining or maintaining approval of AMX0035 for the treatment of ALS, AD and the other indications for which we are developing AMX0035, could adversely affect our efforts to seek approval from regulatory authorities for AMX0035 in other jurisdictions or potential indications.

AMX0035 is a fixed-dose combination drug product and certain regulatory authorities, including the FDA, 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 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. To satisfy these requirements, the FDA typically requires a clinical factorial study, designed to assess the effects attributable to each drug in the combination product. This is particularly true when the ingredients are directed at the same sign or symptom of the disease or condition.

The FDA has accepted a variety of approaches to satisfy the combination rule. In December 2015, the FDA proposed regulations that would allow the agency to waive the requirements of the combination rule for certain drug products under particular circumstances. The FDA has not, to date, finalized these regulations, but the FDA has stated that factorial studies may be unethical (e.g., omitting a drug known to improve survival) or impractical (there may be too many components to conduct a factorial study, meaning the trial cannot be conducted). The FDA has also stated that it may be possible to use other types of clinical and nonclinical data and

mechanistic information available to demonstrate the contributions of the individual active ingredients to the effect of the combination.

We submitted existing preclinical and clinical data for PB and TURSO (also known as TUDCA), in our NDA submission and the FDA has indicated it will assess the sufficiency of this information during our NDA review period. If the FDA disagrees with our data and rationale, it may not approve our NDA for the treatment of ALS, or may require the successful completion of and data from our ongoing Phase 3 PHOENIX trial before issuing an approval decision. Even if the FDA agrees with our data and rationale, there can be no guarantee that the FDA will issue an approval decision with respect to our NDA submission. Additionally, we will be required to separately satisfy the fixed-dose combination rule for AMX0035 for the treatment of any other indications we pursue in advance of approval. We have only submitted preclinical data to demonstrate the clinical effects of each component in AMX0035 in our NDA and MAA. There can be no assurance that the FDA or the EMA will conclude that our preclinical data are sufficient for these purposes or, even if they are, that the results from our preclinical studies demonstrate the clinical effects of each component in AMX0035.

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. If the FDA, the EMA or other comparable foreign regulatory authorities require us to conduct one or more clinical trials, 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 concentrated our research and development efforts on the treatment of neurodegenerative and central nervous system disorders, a field that has seen very limited success in product development.

We have focused our research and development efforts on addressing neurodegenerative and CNS disorders. 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, AD and other neurodegenerative disorders. Our future success is highly dependent on the successful development and commercialization of AMX0035 and any future product candidates for treating neurodegenerative and CNS disorders. Developing and commercializing AMX0035 and any future product candidates for treatment of neurodegenerative and CNS disorders 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 Health Canada, the FDA, the EMA and other comparable foreign regulatory authorities.

The regulatory approval processes of the FDA, Health Canada, the EMA and other comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain or maintain regulatory approval for AMX0035 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 United States, Canada, or the EU without obtaining regulatory approval from the FDA, Health Canada, or the EMA, respectively. Regulatory authorities in other jurisdictions may have similar requirements. The time required to obtain approval by the FDA, Health Canada, the EMA 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. In June 2022, we obtained marketing authorization with conditions for AMX0035 (ALBRIOZA) in Canada. While we have received marketing authorization with conditions from Health Canada, and have submitted an NDA to the FDA and an MAA to the EMA, to date, we have not submitted any other similar drug approval submissions to comparable foreign regulatory authorities for AMX0035 or any other product candidate. We have not obtained approval of our NDA from the FDA or our MAA from the EMA, and there can be no assurance that we will receive such approvals. One of the conditions of the marketing authorization in Canada is the provision of data from our ongoing PHOENIX trial. There is no guarantee that Health Canada will accept the data from our PHOENIX trial as satisfying the conditions and grant a marketing authorization without conditions for ALBRIOZA for the treatment of ALS. Health Canada could require Amylyx to make further undertakings with respect to confirmatory clinical trials if it is not satisfied with the data from the PHOENIX trial, in order for AMX0035 to continue to be marketed in Canada. On March 30, 2022, the FDA held a virtual meeting of the Advisory Committee. At that meeting, on the question whether the data from our Phase 2 CENTAUR trial and OLE trial established a conclusion that AMX0035 is effective in the treatment of patients with ALS, the Advisory Committee voted 4 (yes) and

6 (no). The FDA informed us, and formally announced in the Federal Register on August 3, 2022, that the agency will reconvene the Advisory Committee to further discuss the NDA for AMX0035 on September 7, 2022 to focus on reviewing additional analyses of data from our clinical trials that were determined by the FDA to constitute a major amendment to the NDA. Although the FDA considers the recommendations of its advisory committees, the recommendation by the Advisory Committee is non-binding. The final decision regarding approval of a pending NDA is made by the FDA, and we remain committed to pursuing its approval in the U.S. given the pressing need for new treatments for ALS. If we experience delays in obtaining and maintaining regulatory approval or if we fail to obtain or maintain such approvals, the commercial prospects for AMX0035 may be harmed and our ability to generate revenues will be materially impaired.

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 initial and potential additional indications or any 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, Health Canada, the EMA or any other comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. Additionally, our expenses could increase if we are required by the FDA, Health Canada, the EMA 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. It is possible that even if AMX0035 or any 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 or any 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 or any future product candidate, or mistakenly believe that AMX0035 or any future product candidates are toxic or not well-tolerated when that is not in fact the case.

AMX0035 and any of our future product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA, Health Canada, the EMA 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, Health Canada, the EMA or other comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication 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, Health Canada, the EMA 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, Health Canada, the EMA and comparable authorities in other countries may disagree with our interpretation of data from clinical trials or preclinical studies and may require additional trials or studies to support marketing approval;
- the data collected from clinical trials of AMX0035 or any 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 Canada, the United States, the EU or elsewhere;
- the FDA, Health Canada, the EMA 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, Health Canada, the EMA 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 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. The FDA, Health Canada, the EMA 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 or any future product candidates are promising, such data may not be sufficient to support approval by the FDA or any other regulatory authority. Although we believe that the results of our CENTAUR trial demonstrate that

the administration of AMX0035 resulted in a statistically significant improvement of both long-term function, as measured by the ALSFRS-R score, and survival (based on a longer-term analysis of CENTAUR patients), regulatory authorities may disagree with the interpretation of our data. For example, at the Advisory Committee meeting on March 30, 2022, the FDA noted a number of concerns that, in the FDA's view, may affect the interpretability of the results from the CENTAUR trial.

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. The FDA has interpreted this evidentiary standard to require typically at least two adequate and well-controlled clinical investigations to establish effectiveness of a drug product, although under certain circumstances the FDA has indicated that a single multi-center trial with certain characteristics, or one adequate and well-controlled trial with confirmatory evidence, may also satisfy this standard. Nonetheless, the FDA generally requires two adequate and well controlled Phase 3 clinical trials demonstrating safety and efficacy before granting marketing approval of a drug product. Accordingly, there is no guarantee that the FDA will grant marketing approval to AMX0035 on the basis of the CENTAUR trial. Even though the FDA has accepted our NDA submission for review, the FDA has extended its review time for our NDA and may still issue a Complete Response Letter if it otherwise deems our NDA submission to be deficient for approval purposes, which would delay our plans for commercialization even if we are not required to conduct additional trials.

There can be no assurance that the FDA and other regulatory agencies, including Health Canada and the EMA, will not require additional clinical trials to support an application for the use of AMX0035 in the treatment of ALS or any other indication. This may be the case particularly as these regulatory authorities may consult with one another or as we may be required to apprise the respective agencies of studies we are conducting of AMX0035 for ALS in conjunction with our requests for marketing approval or in response to requests and updates from the respective agency. In July 2022, we received approval of AMX0035 from Health Canada, with conditions, for the treatment of ALS. One of the conditions of the approval is the provision of data from our ongoing PHOENIX trial. There is no guarantee that Health Canada will accept the data from our PHOENIX trial and grant full approval for AMX0035 for the treatment of ALS Additionally, the EMA may also find that our CENTAUR trial, together with any data from our global Phase 3 PHOENIX trial that may be provided during or after the review period for these applications, is not sufficient to support our request for marketing authorization in those jurisdictions. It is typically the case not just in the United States, but also in Canada and the EU, that marketing approvals are based on two Phase 3 clinical studies.

In addition, disruptions caused by the COVID-19 pandemic 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 toward the COVID-19 pandemic. 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 COVID-19, 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 the ongoing COVID-19 pandemic. As a result of the COVID-19 pandemic, we may face delays in meeting our anticipated timelines for our ongoing and planned clinical trials.

Additionally, as of May 26, 2021, the FDA noted it is continuing to ensure timely reviews of applications for medical products during the ongoing COVID-19 pandemic in line with its user fee performance goals and conducting mission critical domestic and foreign inspections to ensure compliance of manufacturing facilities with FDA quality standards. However, the FDA may not be able to continue its current pace and approval timelines could be extended, including where a pre-approval inspection or an inspection of clinical sites is required and due to the ongoing COVID-19 pandemic and travel restrictions the FDA is unable to complete such required inspections during the review period. Since March 2020, when foreign and domestic inspections were largely placed on hold due to the COVID-19 pandemic, the FDA has been working to resume routine surveillance, bioresearch monitoring and pre-approval inspections on a prioritized basis. Since April 2021, the FDA has conducted limited inspections and has employed remote interactive evaluations, using risk management methods, to meet user fee commitments and goal dates. Ongoing travel restrictions and other uncertainties continue to impact oversight operations both domestic and abroad and it is unclear when standard operational levels will resume. The FDA is continuing to complete mission-critical work, prioritize other higher-tiered inspectional needs (e.g., for-cause inspections), and carry out surveillance inspections using risk-based approaches for evaluating public health. Should the FDA determine that an inspection is necessary for approval and an inspection cannot be completed during the review cycle due to restrictions on travel, and the FDA does not determine a remote interactive evaluation to be adequate, the agency has stated that it generally intends to issue, depending on the circumstances, a complete response letter or defer action on the application until an inspection can be completed. Further, if there is inadequate information to make a determination on the acceptability of a facility, the FDA may defer action on the application until an inspection can be completed. During the COVID-19 pandemic, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic and may experience delays in their regulatory activities. For example, with respect to new sites or facilities

in the EEA, which have never had a current Good Manufacturing Practices ("cGMP") inspection or authorization, the EMA has stated that a distant assessment may be conducted in order to evaluate if the site could be authorized without an on-site pre-approval inspection. If an approval is granted, it should be indicated that the certificate has been granted on the basis of a distant assessment and an on-site inspection should be conducted when circumstances permit. If a cGMP certificate cannot be granted as a result of the distant assessment, a clock-stop in the regulatory approval process will be imposed until an on-site inspection is possible. In addition, even if we were to obtain approval, regulatory authorities may approve AMX0035 or any 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 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 or any future product candidates.

In Canada, pre-approval GMP inspections are not performed in association with the NDS. Instead, Health Canada relies on a Drug Establishment License ("DEL") to determine the site's compliance with GMP. DELs can only be held by companies in Canada, and that company becomes the importer of record for the drug. To import, the sites of manufacture, testing and packaging of the Drug Substance and Drug Product are required to be listed on the DEL. Listing is dependent on having an inspection report from a recognized sister regulatory agency such as the EMA or the FDA. As a result of the COVID-19 pandemic, inspection reports can now be up to three years old. The site of manufacture of the drug product for AMX0035 is in Canada and is subject to routine inspections from Health Canada. These Canadian inspections are currently being performed remotely as a result of the COVID-19 pandemic.

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

To obtain regulatory approval to commercialize AMX0035 and any 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.

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 or any future product candidates we develop, including:

- regulators, or institutional review boards ("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 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 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 or any 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 or any future product candidate or other materials necessary to conduct clinical trials of AMX0035 or any 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 Health Canada, the FDA, the EMA 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.

Negative or inconclusive impressions of the results from our earlier clinical trials of AMX0035 for the treatment of ALS or AD, or any other clinical trial or preclinical studies in animals that we 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 initial or potential additional indications, or any future product candidate. If later stage clinical trials, including our ongoing global Phase 3 PHOENIX trial in ALS, do not produce favorable results with very strong statistical significance, our ability to obtain or maintain any prior-issued regulatory approval for AMX0035 for ALS (including our marketing authorization with conditions from Health Canada) or potential additional indications, or any future product candidate, may be adversely impacted.

Our failure to successfully initiate and complete clinical trials of AMX0035 for ALS, AD or potential additional indications and to demonstrate the efficacy and safety of AMX0035, including each component thereof, necessary to obtain and maintain regulatory approval to market AMX0035 would significantly harm our business. 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 or any 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 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 AMX0035 for our initial or potential additional indications as well as for 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 Health Canada, the FDA, the EMA and other regulatory authorities in the United States 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 marketing authorization with conditions, for AMX0035 (ALBRIOZA) in Canada 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 expect to 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 biologic 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 United States, Canada, EU 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, Health Canada, the EMA 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. For example, during the review of our NDA, the FDA has requested clarifying information regarding our preclinical and clinical data. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay,

limit, or prevent marketing approval of a product candidate. For example, based on dialogue with the FDA, we made our NDA submission to the FDA without including data from our ongoing Phase 3 PHOENIX trial; however, the FDA or other regulatory authorities may disagree with our data or rationale, or both, and thus may not approve our NDA for the treatment of ALS, or may require completion of our ongoing Phase 3 PHOENIX global clinical trial prior to issuing an approval decision for our marketing applications for AMX0035. 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. On March 30, 2022, the FDA held a virtual meeting of the Advisory Committee. At that meeting, on the question whether the data from our CENTAUR trial and OLE trial established a conclusion that AMX0035 is effective in the treatment of patients with ALS, the Advisory Committee voted 4 (yes) and 6 (no). The FDA informed us, and formally announced in the Federal Register on August 3, 2022, that the agency will reconvene the Advisory Committee to further discuss the NDA for AMX0035 on September 7, 2022 to review additional analyses of data from our clinical trials that were determined by the FDA to constitute a major amendment to the NDA. The outcome of this meeting and the March 30, 2022 meeting could materially harm the outcome of the FDA's review of our application and lead to a complete response letter. Additionally, in July 2022 we received marketing authorization with conditions of AMX0035 (ALBRIOZA) from Health Canada for the treatment of ALS. One of the conditions of the marketing authorization in Canada is the provision of data from our ongoing PHOENIX trial. There is no guarantee that Health Canada will accept the data from our PHOENIX trial as satisfying the conditions and grant a marketing authorization without conditions for ALBRIOZA for the treatment of ALS. Health Canada could require Amylyx to make further undertakings with respect to confirmatory clinical trials if it is not satisfied with the data from the PHOENIX trial, in order for AMX0035 to continue to be marketed in Canada. As such, we may be unable to obtain the marketing approvals we are pursuing and any marketing approvals we ultimately obtain, including any conditional approvals, may be denied, limited or subject to restrictions or post-approval commitments that could render the approved product not commercially viable.

If we experience delays in obtaining and maintaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates, including for AMX0035 in other indications, 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 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 or any future product candidates. In addition, the clinical results seen in the CENTAUR trial may not be repeated 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 may 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 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 or any 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 Health Canada, the FDA, the EMA or other comparable foreign regulatory authorities. Additionally, certain clinical trials for AMX0035 and any 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 ALS, 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 or any future product candidates. Even once enrolled, we may be unable to retain a sufficient number of patients to complete any of our trials. For example, ALS patients have significant mobility issues, morbidities and other complications that have historically made retention in ALS trials, more challenging. These challenges are also present with many other neurodegenerative indications, including indications for which we may run clinical trials in the future. In the past, we have had discontinuations in our clinical trials, including in our CENTAUR trial and CENTAUR OLE trial. Discontinuations may occur in the future and could result in delays 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 neurological diseases we target are rare.

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 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. 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 ALS, AD and additional indications and any future product candidates, or could render further development impossible. For example, the impact of public health epidemics, such as the ongoing COVID-19 pandemic, may delay or prevent patients from enrolling or from receiving treatment in accordance with the protocol and the required timelines, which could delay our clinical trials, or prevent us or our partners from completing our clinical trials at all, and harm our ability to obtain and maintain approval for such product candidate. 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 the COVID-19 pandemic and related illness or actions taken to slow the spread of COVID-19 or otherwise, the integrity of data from our clinical trials may be compromised or not accepted by Health Canada, the FDA, the EMA 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 or any 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, if approved in the United States or EU, we intend to commercialize a different formulation of AMX0035 from the formulation evaluated in the CENTAUR trial. This change may lead the FDA and other 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 of AMX0035 evaluated in the CENTAUR trial. Should we have to conduct comparability testing to bridge earlier clinical data obtained from AMX0035 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 or any future product candidates and jeopardize our ability to commence sales and generate revenue.

AMX0035 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, 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 or denial of regulatory approval by the FDA, Health Canada, the EMA or comparable foreign regulatory authorities. Results of our 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, nausea, constipation, headache, fatigue, proteinuria, and decreased appetite. Health Canada has additionally identified hypersalivation as an additional treatment-emergent adverse event in need of being addressed. However, there can be no guarantee that we would observe a similar tolerability profile of AMX0035 in our ongoing global Phase 3 PHOENIX clinical trial or in other future clinical trials. 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.

If unacceptable side effects arise in the development of AMX0035 or any future product candidates, we, the FDA, Health Canada, the EMA 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 or any 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 could adversely affect enrollment in clinical trials, regulatory approval and commercialization of AMX0035 in other indications. Additionally, there may be negative findings regarding components of AMX0035 or future product candidates by other parties. For example, Humanitas is conducting a Phase 3 clinical trial in the EU to assess the safety and efficacy of TURSO in patients with ALS which may lead to additional findings as to the safety profile of TURSO. Any negative findings by third parties may impact the future approvability or labeling of AMX0035 or other product candidates we may develop. In addition, side effects may not be appropriately recognized or managed by the treating medical staff. We have no relationship with Humanitas. If their Phase 3 clinical trial is successful and TURSO is approved by the FDA or any other regulatory agency, TURSO may become a commercialized product competitive with AMX0035, unless our intellectual property protections and any regulatory exclusivities we possess or may possess in the future prevent such commercialization. Inadequate training in recognizing or managing the potential side effects of AMX0035 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, including our ongoing global Phase 3 PHOENIX trial, or limit its commercial adoption.

Moreover, clinical trials of AMX0035 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 or a future product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects.

Finally, 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 were to receive marketing approval by the FDA or the EMA or be commercialized, we would continue to be subject to the risks that the FDA, EMA or similar 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.

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

We are developing AMX0035 for the treatment of ALS, AD 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. On March 18, 2022, we launched an FDA-authorized expanded access program in the United States for AMX0035 for certain adults with ALS; however, this expanded access program still may not reach all ALS patients.

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 the Accelerating Access to Critical Therapies for ALS Act and prior "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 expanded access programs and the former of which is intended to support research and development related to ALS, specifically. A possible consequence of both activism and legislation in this area may be the need for us to initiate an expanded access program 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 March 2022, we announced the launch of our U.S. expanded access program for AMX0035 in patients who meet program eligibility requirements. We are a small company with limited resources and unanticipated trials or access programs could result in diversion of resources from our primary goals.

In addition, some patients who receive access to drugs prior to their commercial approval through compassionate use, expanded access programs 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 our U.S. expanded access program or any other compassionate use and/or expanded access program 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.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our clinical development activities and the diseases AMX0035 is being developed to treat, and we intend to continue to utilize appropriate social media in connection with our commercialization efforts for ALBRIOZA in Canada and in any other jurisdictions where we obtain regulatory approvals. Social media practices in the biotechnology and biopharmaceutical industries continue to evolve and regulations and regulatory guidance relating to such use are evolving and not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business, resulting in potential regulatory actions against us, along with the potential for litigation related to off-label marketing or other prohibited activities and heightened scrutiny by the FDA, the SEC and other regulators. For example, patients may use social media channels to comment on their experience in an ongoing blinded clinical trial or to report an alleged adverse event. If such disclosures occur, there is a risk that trial enrollment may be adversely impacted, that we may fail to monitor and comply with applicable adverse event reporting obligations or that we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product candidates. There is also a risk of inappropriate disclosure of sensitive or confidential information or negative or inaccurate posts or comments about us on any social networking website. In addition, we may encounter attacks on social media regarding our company, management, AMX0035 or future product candidates. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions or incur other harm to our business.

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

Although the development and commercialization of AMX0035 for the treatment of ALS is our current primary focus, as part of our longer-term growth strategy, we plan to evaluate AMX0035 in other indications and develop other product candidates. We intend to evaluate internal opportunities from AMX0035 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. These other potential product candidates will require additional, time-consuming development efforts prior to commercial sale, including preclinical studies, clinical trials and approval by the FDA, Health Canada, the EMA 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 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 indications and modifications for which to investigate AMX0035 in the future. We may expend our limited resources to pursue a particular indication or formulation for AMX0035 and fail to capitalize on product candidates, indications or formulations 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. As a result, we may fail to generate additional clinical development opportunities for AMX0035 for a number of reasons, including, that AMX0035 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 multiple clinical trials in patients with ALS and other indications, which may make our decision as to which indication to focus on more difficult. As a result, we may forgo or delay pursuit of opportunities with other indications that could have had greater commercial potential or likelihood of success. In addition, we plan to explore the use of AMX0035 in patients with Wolfram syndrome and other indications. However, we may focus on or pursue one or more of our target indications over other potential indications and such development efforts may not be successful, which would cause us to delay the clinical development and approval of AMX0035. Furthermore, research activities to identify additional indications for AMX0035 require substantial technical, financial, and human resources. We may not successfully develop these additional modifications for chemistry-related, stability-related, or other reasons. 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 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. Identifying, selecting and acquiring promising product candidates requires substantial technical, financial, and human resources expertise. Efforts to do so may not result in the actual acquisition or license of a particular product candidate, potentially resulting 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 current or future indications. 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 may be adversely affected.

The clinical and commercial landscape for the treatment of ALS and other neurodegenerative diseases, including AD is highly competitive and subject to rapid and significant technological change. We face competition with respect to our current indications for AMX0035 and will face competition with respect to any future indications of AMX0035 or other drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, Humanitas Mirasole SpA is currently conducting a Phase 3 clinical trial in the EU to assess the safety and efficacy of TURSO in patients with ALS, which, if approved, may be commercialized as a competitor to AMX0035. If this study meets its clinical endpoints, this monotherapy treatment could be approved by the FDA, the EMA and other regulatory authorities, and TURSO may become a commercialized product competitive with AMX0035, unless our intellectual property protections and any regulatory exclusivities we possess or may possess in the future prevent such commercialization. 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.

Several large pharmaceutical companies market FDA-approved drugs for the treatment of ALS. These drugs include: Riluzole, marketed by Sanofi-Aventis U.S. LLC, and Radicava, marketed by Mitsubishi Tanabe Pharma America, Inc. Additionally, Mitsubishi Tanabe Pharma America, Inc. ("MTPA"), is developing an oral alternative to Radicava. In the first quarter of 2022 the FDA accepted MTPA's application for priority review of its oral alternative to Radicava, with a Prescription Drug User Fee Act ("PDUFA") date of May 12, 2022. Our potential competitors include pharmaceutical and biotechnology companies, such as Biogen, Inc., Orphazyme A/S, Biohaven Pharmaceutical Holding Co Ltd., UCB S.A., Alexion Pharmaceuticals, Inc. and Apellis Pharmaceuticals, Inc., specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions.

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 or any future product candidates obsolete or non-competitive before we can recover development and commercialization expenses. If AMX0035 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 drug products that are more effective or less costly than AMX0035 or any future product candidates that we may develop, which could render such product candidates obsolete and noncompetitive.

Following approval for AMX0035 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. We expect to face competition with respect to our commercialization of ALBRIOZA in Canada and any future product candidate, if approved, in Canada. Following approval by Health Canada, the FDA or the EMA for the commercial sale of AMX0035 or any future product candidate, 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 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 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. For example, even though Health Canada has granted marketing authorization with conditions of AMX0035 (ALBRIOZA), comparable regulatory authorities in the United States, EU and other foreign jurisdictions must also approve the manufacturing, marketing and promotion of AMX0035 in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in Canada, the United States or the EU 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 United States 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.

We have received marketing authorization with conditions for AMX0035 (ALBRIOZA) in Canada and have submitted marketing applications in the United States and the EU. Regulatory authorities in jurisdictions outside of the United States 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 any product candidates approved for sale in any jurisdiction except Canada, and we do not have experience in obtaining regulatory approval in either domestic or 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 or any future product candidates will be harmed.

Even though we have obtained orphan drug designation for AMX0035 for the treatment of ALS in the United States and the EU and for the treatment of Wolfram syndrome in the United States, 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 United States 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 United States, or a patient population of greater than 200,000 people in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the EU, the EMA Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the EU. 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 drug in the EU would be sufficient to justify the necessary investment in developing the drug or biologic 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).

In September 2017, the FDA granted orphan drug status to AMX0035 for the treatment of patients with ALS in the United States, and in June 2020, the EMA granted orphan drug status to AMX0035 for the treatment of patients with ALS in the EU. We also received orphan drug status for AMX0035 for the treatment of patients with Wolfram syndrome in the United States in November 2020. 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 EMA from approving another marketing application for the same drug for that time period. Another drug may receive marketing approval prior to AMX0035. The applicable period is seven years in the United States 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 can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug 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 other similar medicinal products. 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. 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 ALS and Wolfram syndrome, if we receive approval for AMX0035 for a modified or different indication, our current orphan designations 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, 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 if orphan drug exclusivity is granted to AMX0035 if and when it is approved, that exclusivity may not prevent the approval of TURSO by the FDA, the EMA or other regulatory authorities as a monotherapy treatment for ALS 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 would be economically unviable when facing competition to maintain our exclusivity.

We may pursue orphan drug designation for AMX0035 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 United States, or a patient population of greater than 200,000 people in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. 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 for other indications, exclusive marketing rights in the United States 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 received priority review designation for AMX0035 in the United States and may in the future pursue priority review designation for other product candidates that we may develop, but we might not receive such future 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. We have received priority review for AMX0035 and have been assigned, following an FDA review extension, a PDUFA date of September 29, 2022. 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 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 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.

We may seek Breakthrough Therapy Designation by the FDA for a product candidate that we 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.

We may seek Breakthrough Therapy Designation for any product candidate that we 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 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.

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 or any 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 by us and any collaborators in clinical trials, and the sale of AMX0035 in Canada and in other jurisdictions, if approved, in the future, 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 or any 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 or any 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 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 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 $5.0 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 will need to increase our insurance coverage as we commercialize AMX0035 in Canada and other jurisdictions, if approved, or any 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 or any 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 or any 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 or any 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, Health Canada and EMA 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 be subject to periodic unannounced inspections by the FDA, Health Canada and the EMA 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, Health Canada, the EMA 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 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 or any 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 United States and will require us to develop and implement costly compliance programs.

We have operations in Canada and expect to engage in operations in other jurisdictions, including in the United States and EU, as well as other potential jurisdictions, and we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we currently or plan to operate. The Foreign Corrupt Practices Act ("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 United States 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 United States, 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 United States, 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 United States, 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 them on commercially reasonable terms, we may have to alter our development and commercialization plans.

The advancement of AMX0035, and any future product candidates and development programs or activities, as well as the commercialization of ALBRIOZA in Canada and the potential commercialization of AMX0035 in other jurisdictions and of any future product candidates will require substantial additional cash to fund expenses. For some indications of AMX0035 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 Health Canada, the FDA, the EMA 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 or any 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 commercialization activities at our own expense.

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

We may rely on collaborations for the development and commercialization of AMX0035 and any 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 to identify novel drug candidates for neurodegenerative diseases as with our partnership with Sunnybrook Research Institute. 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 or any 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 and any 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 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 or any of our 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 or maintain regulatory approval or successfully commercialize AMX0035 or any 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, may affect 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, the EMA and competent authorities of the EU Member States require us to comply with Good Clinical Practices ("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 the EMA may require us to perform additional clinical trials before approving AMX0035 or any future product candidates, which would delay the regulatory approval process. We cannot be certain that, upon inspection, the FDA or the EMA 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, 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 or any future product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize AMX0035 or any future product candidates. In such an event, our financial results and the commercial prospects for AMX0035 or any 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.

In addition, quarantines, shelter-in-place, and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to the COVID-19 pandemic or other infectious diseases could impact personnel at our CROs, which could disrupt our clinical timelines, which could have a material adverse impact on our business, prospects, financial condition, and results of operations.

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 or any 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 may increase the risk that we will not have sufficient quantities of AMX0035, products, 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, 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 ("APIs") in AMX0035, and for the blending and packaging of AMX0035. Our current strategy is to outsource all manufacturing of AMX0035 and any future product candidates to third parties.

We currently engage third-party manufacturers to provide the APIs of AMX0035 and for the final drug product formulation of AMX0035 that is being used in our clinical trials and for expanded access 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, we may incur added costs and delays in identifying and qualifying any such replacement. In addition, we typically order raw materials and services on a purchase order basis and do not enter into long-term dedicated capacity or minimum supply arrangements with any commercial manufacturer. Moreover, the extent to which the COVID-19 pandemic impacts our ability to procure sufficient supplies for the development of AMX0035, and any future products and product candidates will depend on the severity and duration of the spread of the virus, and the actions undertaken to contain COVID-19 or treat its effects. 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 or any 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 the costs of manufacturing could be prohibitive.
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 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 or any 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 conflict in Ukraine 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, Health Canada, the EMA 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, Health Canada, the EMA 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, or any 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 or any 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 or any future product candidates. If AMX0035 for any of our initial or potential additional indications 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 or any 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 or any future product candidates, operating restrictions and criminal prosecutions, any of which could significantly affect supplies of AMX0035 or any future product candidates. The facilities used by our contract manufacturers to manufacture AMX0035 or any future product candidates must be evaluated by the FDA, Health Canada, the EMA 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, Health Canada, the EMA 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, Health Canada, the EMA or other comparable foreign regulatory authority finds deficiencies or does not approve these facilities for the manufacture of AMX0035 or any 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 or any 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, Health Canada, EMA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop AMX0035 or any future product candidates and market our products, if approved.

The FDA, Health Canada, the EMA and other foreign regulatory authorities require manufacturers to register manufacturing facilities. The FDA, Health Canada, the EMA 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, Health Canada, EMA and comparable foreign regulatory requirements could adversely affect our clinical research activities and our ability to develop AMX0035 or any future product candidates and market our products following approval.

If any third-party manufacturer of AMX0035 or any future product candidates is unable to increase the scale of its production of such product candidates, and/or increase the product yield of its manufacturing, then our costs to manufacture the product may increase and commercialization may be delayed.

In order to produce sufficient quantities to meet the demand for clinical trials, expanded access and, commercialization of AMX0035 in Canada, and any subsequent commercialization of AMX0035 in other jurisdictions, if approved, or any future product candidates that we may develop, our third-party manufacturers will be required to increase their production and optimize their manufacturing processes while maintaining the quality of the product. The transition to larger scale production could prove difficult. In addition, if our third party manufacturers are not able to optimize their manufacturing processes to increase the product yield for AMX0035 or any future product candidates, or if they are unable to produce increased amounts of such product candidates while maintaining the quality of the product, then we may not be able to meet the demands of clinical trials or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operation.

Since the beginning of the COVID-19 pandemic, several vaccines for COVID-19 have received Emergency Use Authorization by the FDA and a number of those later received marketing approval. Additional vaccines may be authorized or approved in the future. The resultant demand for vaccines and potential for manufacturing facilities and materials to be commandeered under the Defense Production Act of 1950, or equivalent foreign legislation, is having rippling effects across the contract manufacturing industry, which may make it more difficult to obtain materials or manufacturing slots for the production needed for our clinical trials and, if approved, our future commercial supply, which could lead to delays in our trials and commercial distribution.

We may need to maintain licenses for active ingredients from third parties to develop and commercialize AMX0035 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 or any 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 Our Intellectual Property

Our commercial success depends on our ability to protect our intellectual property and proprietary technology, and to achieve data and market exclusivities in applicable markets.

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 United States and other countries with respect to our proprietary product candidate, AMX0035, 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 achieve profitability. To protect our proprietary position, we have filed patent applications and may file other patent applications in the United States or abroad related to AMX0035 or any future product candidates that are important to our business; we may also license or purchase patents or patent applications filed by others. 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 United States. Furthermore, patents have a limited lifespan. In the United States, 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 or any future product candidates. In the event that an alternative combination, 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 or any 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 United States, 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 United States 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 United States 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 United States 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 United States 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 United States 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 ("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 United States 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 United States 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 United States. For example, patent laws in various jurisdictions, including jurisdictions covering significant commercial markets, such as the European Patent Office ("EPO"), China and Japan, restrict the patentability of methods of treatment of the human body more than United States 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 or any 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;
- 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 United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the United States 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 United States 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 candidate, AMX0035, 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 United States 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 USPTO or by a court or other trier of fact in the United States, 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 United States 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 United States 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 United States or foreign laws;
- we may not successfully commercialize AMX0035 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 or any 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 information technology systems, but it is possible that these security measures could be breached. In addition, courts outside the United States 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 may be inadequate to protect our competitive position on our products for an adequate amount of time.

Patents have a limited lifespan. In the United States, 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 United States 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 United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a Patent Term Extension ("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 United States. 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 United States, 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.

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

The United States 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 United States 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 United States can be less extensive than

those in the United States. 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 United States and the EU do not afford intellectual property protection to the same extent as the laws of the United States 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 United States and the EU or from selling or importing products made from our inventions in and into the United States 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 or any 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 or any 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 or any future product candidates. If any third-party patents or patent applications are found to cover AMX0035 or any 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 United States 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 or any future product candidates. While we perform periodic searches for relevant patents and patent applications with respect to our proprietary drug candidate, AMX0035, 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 United States and abroad that is relevant to or necessary for the commercialization of AMX0035 or any 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 or any 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 United States, 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 or any 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 United States 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, 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, and any future product candidates. We also expect to collaborate with third parties on the development of AMX0035 and any 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, Employee Matters and Managing Growth

A pandemic, epidemic, or outbreak of an infectious disease, such as the ongoing and evolving COVID-19 pandemic, 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 such as the ongoing and evolving COVID-19 pandemic. The pandemic and policies and regulations implemented by governments in response to the pandemic, often directing businesses and governmental agencies to cease non-essential operations at physical locations, prohibiting certain nonessential gatherings and ceasing non-essential travel have also had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred, supply chains have been disrupted, facilities and production have been suspended, and demand for certain goods and services, such as medical service and supplies, has spiked. We have experienced certain impacts of the COVID-19 pandemic to date, including having to make certain alterations to our preclinical and clinical trial activities, such as scheduling certain work off-site and performing off-site assessments. For example, we had to amend our CENTAUR trial protocol to allow for remote visits by patients, instead of patients making site visits. In addition, in some cases we were forced to delay enrollment at certain sites in our recently completed Phase 2

clinical trial for AMX0035 in AD. There can be no guarantee we will not experience other impacts, 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.

The impact of the COVID-19 pandemic has been and may continue to be extensive in many aspects of society and could continue to result in significant disruptions to the global economy, as well as businesses and capital markets around the world. Although many of the government imposed COVID-19 restrictions have eased, the full extent to which the COVID-19 pandemic could ultimately impact our business, preclinical studies, clinical trials and financial results will depend on future developments, which are highly uncertain and cannot be accurately predicted, including the emergence of new variants and subvariants of the virus that causes COVID-19, such as the Omicron variants and subvariants, for which current vaccinations may be less effective or ineffective, among others. Other global health concerns could also result in social, economic, and labor instability in the countries in which we or the third parties with whom we engage operate.

In response to the COVID-19 pandemic, we have continued to take precautionary measures intended to help minimize the risk of the virus to our employees, including closing or reducing access to our executive offices and temporarily requiring employees to work remotely, suspending all non-essential travel for our employees and discouraging employee attendance at industry events and in-person work-related meetings, all of which could negatively affect our business.

While we have been working closely with our third-party manufacturers, distributors and other partners to manage our supply chain activities and mitigate potential disruptions to the production of AMX0035 as a result of the COVID-19 pandemic, if, despite vaccination efforts, the COVID-19 pandemic continues to cause societal and commercial disruption for an extended period of time, there could be significant and material disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of AMX0035 and any future product candidates. Any such supply disruptions, including disruptions in procuring items that are essential for our research and development activities and securing manufacturing slots for the products needed for such activities, could adversely impact our ability to initiate and complete preclinical studies or clinical trials and generate sales of and revenue from our product candidates, if approved, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

The COVID-19 pandemic has affected and may in the future affect employees of third-party CROs located in affected geographies that we rely upon to carry out our clinical trials. If current efforts to control the COVID-19 pandemic are not successful, if the spread of the virus or any variant of the virus increases, or if a new variant, virus or pandemic emerges, we may experience additional disruptions that could severely impact our business and clinical trials, including:

- delays or difficulties in our commercialization efforts;
- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of sites or facilities serving as our clinical trial sites and staff supporting the conduct of our clinical trials, including our trained therapists, or absenteeism that reduces site resources;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal, state or national governments, employers and others or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;
- risk that participants enrolled in our clinical trials will acquire COVID-19 or another virus or illness while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events or patient withdrawals from our trials;
- limitations in employee resources that would otherwise be focused on conducting our clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;
- delays in receiving authorizations from regulatory authorities to initiate our future clinical trials;
- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials;
- interruption in global shipping that may affect the transport of clinical trial materials, such as the AMX0035 used in our clinical trials;

- changes in local regulations as part of a response to the COVID-19 pandemic or other pandemic which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or the discontinuation of the clinical trials altogether;
- interruptions or delays in preclinical studies due to restricted or limited operations at research and development laboratory facilities;
- delays in necessary interactions with local regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government employees; and
- refusal of the FDA, Health Canada, the EMA or the other regulatory bodies to accept data from clinical trials in affected geographies outside the United States, Canada or the EU or other relevant local geographies.

Any negative impact the COVID-19 pandemic or any future pandemic or similar disruption has on patient enrollment or treatment, or the development of AMX0035 and any 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 and any future product candidates, if approved, increase our operating expenses, which could have a material adverse effect on our financial results. The COVID-19 pandemic has also in the past caused significant volatility in public equity markets and disruptions to the United States 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 renewed shutdowns or other 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 the COVID-19 pandemic or 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.

We are currently operating in a period of economic uncertainty and capital markets disruption, which has been significantly impacted by geopolitical instability, an ongoing military conflict between Russia and Ukraine, and record inflation, any of which could have a material adverse effect on our business, financial condition and results of operations.

U.S. and global markets are experiencing volatility and disruption following the escalation of geopolitical tensions and the military conflict between Russia and Ukraine. In February 2022, a full-scale military invasion of Ukraine by Russian troops began. 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 has contributed to record inflation globally. We are continuing to monitor inflation, the situation in Ukraine and global capital markets and assessing its potential impact on our business, including the impact on the supply chains we rely on for the manufacture of AMX0035 or other future product candidates.

Although, to date, our business has not been materially impacted by the ongoing military conflict between Russian and Ukraine, 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 conflict in Ukraine, 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, including Josh Cohen and Justin Klee, our Co-Chief Executive Officers, James Frates, our Chief Financial Officer, Margaret Olinger, our Global Head of Commercial and Chief Commercial Officer, and Patrick Yeramian, our Global Head of Clinical Research & Development and Chief Medical Officer. We have entered into employment agreements with Mr. Cohen, Mr. Klee, Mr. Frates, Ms. Olinger and Dr. Yeramian, 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. 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 and commercialize AMX0035 or any future product candidates will be limited.

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

As of June 30, 2022, we had 226 full-time employees. Our focus on the development of AMX0035 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 AMX0035 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.

We expect to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We currently expect to continue to significantly increase the number of our employees and the scope of our operations, particularly in the areas of regulatory affairs and sales, marketing and distribution, as well as to support our public company operations. To manage these growth activities, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Our management may need to devote a significant amount of its attention to managing these growth activities. Moreover, our expected growth could require us to relocate to a different geographic area of the country. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion or relocation of our operations, retain key employees, or identify, recruit and train additional qualified personnel. Our inability to manage the expansion or relocation of our operations effectively may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. See "—We previously identified material weaknesses in our internal control over financial reporting. If we fail to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations, meet our reporting obligations or prevent fraud, and investor confidence in our company and the market price of our common stock may be materially and adversely affected." Our expected growth could also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy, including the successful commercialization of AMX0035 or any future product candidates.

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. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this Quarterly Report on Form 10-Q, 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 and any future product candidates, or changes in the development status of our current and any future product candidates;
- any additional regulatory submissions for AMX0035 or any 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 and any future product candidates;
- changes in laws or regulations applicable to AMX0035 and any future product candidates, including but not limited to clinical trial requirements for approvals;
- the failure to obtain coverage and adequate reimbursement of AMX0035 and any 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 and any future product candidates;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of AMX0035 and any 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;
- our ability to effectively manage our growth;
- 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 the COVID-19 pandemic, historically high inflation, rising interest rates and the evolving conflict in Ukraine; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and biopharmaceutical 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, declines in economic growth, increases in the rate of inflation, increases in unemployment rates and uncertainty about economic stability, including most recently in connection with the ongoing and evolving COVID-19 pandemic and the conflict in Ukraine. 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 situation in the Ukraine. 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 an emerging growth company and a smaller reporting company, and the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company until December 31, 2027, although circumstances could cause us to lose that status earlier, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act or if we have total annual gross revenue of $1.07 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than $1.0 billion in non-convertible debt during any three year period before that time, in which case we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company", which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Investors may find our common stock less attractive because we may rely on these exemptions. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter. 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.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This provision allows an emerging growth company to delay the adoption of accounting standards that have different effective dates for public and private companies until those standards would otherwise apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards, and therefore we will not be subject to the same requirements to adopt new or revised accounting standards as other public companies that are not emerging growth companies.

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 August 8, 2022, we had outstanding 58,533,226 shares of common stock, which may be resold in the public market immediately without restriction, unless purchased by our affiliates or existing stockholders subject to a lock-up agreement. Moreover, holders of an aggregate of 45,227,883 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 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 owned more than 5% of our outstanding common stock, own a significant portion of our common stock. As a result, these stockholders acting together, would 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, would 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 certificate of incorporation and 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 amended and restated certificate of incorporation and amended and restated 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 United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under 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.

As a result of becoming a public company, we are obligated to develop and maintain proper and effective internal controls over financial reporting and any failure to maintain the adequacy of these internal controls may adversely affect investor confidence in our company and, as a result, the value of our common shares.

We will be required, pursuant to Section 404 of the Sarbanes Oxley Act ("Section 404"), to furnish a report by management on, among other things, the effectiveness of our internal controls over financial reporting for the fiscal year ending December 31, 2023. This assessment will need to include disclosure of any material weaknesses identified by our management in our internal controls over financial reporting. Our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting until our first annual report required to be filed with the SEC following the date we are no longer an emerging growth company, as defined in the JOBS Act, and are not a smaller reporting company with less than $100 million in annual revenue. At such time as we are required to obtain auditor attestation, if we then have a material weakness, we would receive an adverse opinion regarding our internal control over financial reporting from our independent registered public accounting firm. We are required to disclose significant changes made in our internal controls procedures on a quarterly basis.

We continue the costly and challenging process of enhancing our financial reporting systems and processes as necessary to allow for the operation of effective internal controls over financial reporting to comply with the requirements of Section 404. We may not be able to complete our assessment, testing and any required remediation of internal controls over financial reporting in a timely fashion. Our compliance with Section 404 will require that we incur substantial legal, accounting and other compliance expense and expend significant management efforts. We currently do not have an internal audit group. We will need to hire additional accounting and finance personnel and consultants with appropriate public company experience and technical accounting knowledge to develop and maintain the internal controls over financial reporting necessary to comply with Section 404.

We have identified past material weaknesses in our internal controls over financial reporting. If during the evaluation and testing of our internal controls over financial reporting, we identify one or more additional material weaknesses in future periods, we will be unable to assert that our internal controls over financial reporting are effective. We cannot assure you that there will not be additional material weaknesses in our internal controls over financial reporting in the future. Any failure to maintain effective internal controls over financial reporting could severely inhibit our ability to accurately report our financial condition or results of operations. If we are unable to conclude that our internal controls over financial reporting are effective, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common shares could decline, and we could be subject to sanctions or investigations by the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal controls over financial reporting, or to implement or maintain other effective control systems required of public companies, could also negatively impact our ability to access to the capital markets.

In addition, effective disclosure controls and procedures enable us to make timely and accurate disclosure of financial and non-financial information that we are required to disclose. As a public company, if our disclosure controls and procedures are ineffective, we may be unable to report our financial results or make other disclosures accurately and on a timely basis, which could cause our reported financial results or other disclosures to be materially misstated and result in the loss of investor confidence and cause the market price of our common shares to decline.

We previously identified material weaknesses in our internal control over financial reporting. If we fail to implement and maintain an effective system of internal controls, we may be unable to accurately report our results of operations, meet our reporting obligations or prevent fraud, and investor confidence in our company and the market price of our common stock may be materially and adversely affected.

Prior to the completion of our IPO, we had limited accounting personnel, IT personnel and other resources with which to address internal controls over financial reporting. In connection with the audits of our consolidated financial statements as of and for the years ended December 31, 2020 and 2019, we identified two material weaknesses in our internal controls over financial reporting. As defined in the standards established by the U.S. Public Company Accounting Oversight Board a “material weakness” is a deficiency,

or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the annual or interim financial statements will not be prevented or detected on a timely basis. We have concluded that these material weaknesses have been remediated, but there can be no assurance that we will not identify future material weaknesses or reportable conditions.

Deficiencies in our internal controls over financial reporting that were considered to be a material weakness as of December 31, 2019 were related to the lack of a sufficiently precise review over both valuations prepared by our third-party valuation experts as well as the completeness of operating expenses. We remediated the material weakness related to the review of valuation reports by adding a precise review control that was performed by our accounting personnel with the appropriate technical expertise to review valuation reports. In addition, we have hired an accounting executive with the requisite knowledge in the application of U.S. GAAP and SEC reporting who will be collaborating and reviewing valuation reports prepared by our third-party valuation experts.

Further deficiencies in our internal controls over financial reporting that were considered to be a material weakness related to deficiencies in the design of controls over the expenditures process. Specifically, our information technology controls related to the expenditures cycle were not designed to post invoices approved in the correct period, and our controls over the review of the completeness of operating expenses as it related to our close cycle were not appropriately designed, as we lacked sufficient personnel in our Finance and IT organizations to review and provide reasonable assurance that transactions were being recorded timely and completely. We implemented changes to our internal controls over financial reporting to remediate this material weakness which included hiring a sufficient number of accounting and IT personnel to focus on our information technology systems and to adequately manage the monthly close process.

We are subject to the Sarbanes-Oxley Act. Section 404 requires that we include a report from management on the effectiveness of our internal control over financial reporting in our annual report on Form 10-K beginning with our second annual report on Form 10-K after becoming a public company. In addition, our reporting obligations may place a significant strain on our management, operational and financial resources and systems for the foreseeable future. We may be unable to timely complete our evaluation testing and any required remediation.

During the course of documenting and testing our internal control procedures, in order to satisfy the requirements of Section 404, we may identify other weaknesses and deficiencies in our internal control over financial reporting. If we fail to maintain the adequacy of our internal control over financial reporting, as these standards are modified, supplemented or amended from time to time, we may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting in accordance with Section 404.

Generally speaking, if we fail to achieve and maintain an effective internal control environment, it could result in material misstatements in our financial statements and could also impair our ability to comply with applicable financial reporting requirements and related regulatory filings on a timely basis. As a result, our businesses, financial condition, results of operations and prospects, as well as the trading price of our common stock, may be materially and adversely affected.

Additionally, ineffective internal control over financial reporting could expose us to increased risk of fraud or misuse of corporate assets and subject us to potential delisting from the stock exchange on which we list, regulatory investigations and civil or criminal sanctions. We may also be required to restate our financial statements from prior periods.

Our bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America 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 certificate of incorporation or bylaws;
- any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated 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 amended and restated certificate of incorporation further provides that the federal district courts of the United States of America 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 amended and restated 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 amended and restated 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 or any 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 IPO.

Pursuant to our 2022 Stock Option and Incentive Plan (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 will 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 that we did not incur as a private 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 establishment and 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 ("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. Emerging growth companies and smaller reporting companies are exempted from certain of these requirements, but we may be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. 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.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. 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 information technology 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 information technology ("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, spamming or other means, and deliberate attacks and attempts to gain unauthorized access to computer systems and networks, have increased in frequency and sophistication. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient. 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. Any cyber-attack, data breach or destruction 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 United States 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 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 and may incur significant additional expense to implement further data protection measures.

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, 2021, we had U.S. federal and state net operating loss carryforwards of $115.7 million and $102.9 million, respectively, some of which begin to expire in 2034. As of December 31, 2021 and 2020, we also had U.S. federal research and development tax credit carryforwards of $2.7 million and $1.6 million, respectively, which begin to expire in 2029. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future taxable income or tax liabilities, respectively. U.S. federal and certain state net operating losses generated in taxable years beginning after December 31, 2017 are not subject to expiration. Federal net operating losses generally may not be carried back to prior taxable years except that, under the Coronavirus Aid, Relief and Economic Security Act, federal net operating losses generated in 2018, 2019 and 2020 may be carried back to each of the five taxable years preceding the taxable year in which the loss arises. Additionally, for taxable years beginning after December 31, 2020, the deductibility of federal net operating losses generated in taxable years beginning after December 31, 2017 is limited to 80% of our taxable income in such taxable year.

In general, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the "Code"), 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 net operating loss carryforwards or tax credits, or NOLs or credits, 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. Our existing federal and state NOLs and our existing research and development credits may be subject to limitations arising from previous ownership changes and, if we undergo an ownership change, our ability to utilize NOLs or credits could be further limited by Sections 382 and 383 of the Code. We have not yet completed a Section 382 analysis. In addition, this offering or 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 Code. Our NOLs or credits may also be impaired under state law. Accordingly, we may not be able to utilize a material portion of our NOLs or credits. Furthermore, our ability to utilize our NOLs or credits is conditioned upon our attaining profitability and generating U.S. federal and state taxable income. As described above, we have incurred significant net losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future; and therefore, we do not know whether or when we will generate the U.S. federal or state taxable income necessary to utilize our NOLs or credits that are subject to limitation by Sections 382 and 383 of the Code.

We may not be entitled to forgiveness of our loan under the Paycheck Protection Program of the Coronavirus Aid, Relief, and Economic Security Act, and our application for the loan could in the future be determined to have been impermissible or could result in damage to our reputation.

In April 2020, we received proceeds of $0.3 million from a loan (the "PPP Loan") under the Paycheck Protection Program (the "PPP") of the Coronavirus Aid, Relief, and Economic Security Act ("CARES Act"), a portion of which may be forgiven, which we have used to retain employees, maintain payroll and make lease and utility payments. The PPP Loan had a maturity date of April 19, 2022 and an annual interest rate of 1.0%. Payments of principal and interest on the PPP Loan were originally deferred for the first six months of the term. Thereafter, we were required to pay the lender equal monthly payments of principal and interest. Under the PPP, we applied for and were granted forgiveness for the entirety of the PPP Loan. The amount of loan proceeds eligible for forgiveness was originally based on a formula that takes into account a number of factors, including the amount of loan proceeds used by us during the eight-week period after the loan origination for certain purposes, including payroll costs, interest on certain mortgage obligations, rent payments on certain leases, and certain qualified utility payments, provided that at least 75% of the loan amount was used for eligible payroll costs. Subject to the other requirements and limitations on loan forgiveness, only loan proceeds spent on payroll and other eligible costs during the covered eight-week period qualified for forgiveness.

In order to apply for the PPP Loan, we were required to certify, among other things, that the current economic uncertainty made the PPP Loan request necessary to support our ongoing operations. We made this certification in good faith after analyzing, among other things, our financial situation and access to alternative forms of capital, and believe that we satisfied all eligibility criteria for the PPP Loan, and that our receipt of the PPP Loan is consistent with the broad objectives of the PPP. The certification described above does not contain any objective criteria and is subject to interpretation. If, despite our good-faith belief that given our circumstances we satisfied all eligible requirements for the PPP Loan, we are later determined to have violated any of the laws or governmental regulations that apply to us in connection with the PPP Loan, such as the FCA, or it is otherwise determined that we were ineligible to receive the PPP Loan, we may be subject to penalties, including significant civil, criminal and administrative penalties and could be required to repay the PPP Loan in its entirety. In addition, receipt of a PPP Loan may result in adverse publicity and damage to reputation, and a review or audit by the U.S. Small Business Administration or other government entity or claims under the FCA could consume significant financial and management resources. Notwithstanding the forgiveness of the PPP Loan, on October 7, 2021, we repaid the PPP Loan in full.

We could be subject to securities class action litigation.

In the past, 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 biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it 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, they 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 may 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 and Use of Proceeds.

Use of Proceeds from Our Initial Public Offering

In January 2022, we completed our initial public offering in which we issued and sold 11,369,369 shares of common stock at a price to the public of $19.00 per share, for gross proceeds of $216.0 million and net proceeds of approximately $196.4 million after deducting underwriting discounts and commissions and other offering expenses. None of the expenses associated with the initial public offering were paid to directors, officers, persons owning 10% or more of any class of our equity securities, or to their associates, or to our affiliates. The offer and sale of our shares were registered pursuant to a Registration Statement on Form S-1 (Registration No. 333-261703) and the related Registration Statement on Form S-1 (Registration No. 333-262046) filed pursuant to Rule 462(b) under the Securities Act, which were declared effective on January 6, 2022 (together, the "Registration Statement"). Goldman Sachs & Co. LLC, SVB Leerink LLC and Evercore Group L.L.C. acted as lead book-running managers. H.C. Wainwright & Co., LLC acted as co-manager for the initial public offering. Shares of our common stock began trading on The Nasdaq Global Market on January 7, 2022.

There has been no material change in the planned use of proceeds from our initial public offering as described in our Prospectus that forms a part of our Registration Statement, which was filed with the SEC pursuant to Rule 424 on January 10, 2022. As of June 30, 2022, we consumed approximately $90.6 million of net proceeds from the initial public offering, primarily to advance AMX0035 through clinical trials, manufacture drug supply, prepare for potential commercialization and for working capital and general corporate purposes. We invested the remaining funds received in cash equivalents and other marketable securities in accordance with our investment policy.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

Exhibit Number	Description
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 Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
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.
# Indicates a management contract or any compensatory plan, contract or arrangement.

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 PHARMCEUTICALS, INC.

Date: August 11, 2022

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

By: /s/ 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: August 11, 2022

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: August 11, 2022

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: August 11, 2022

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 June 30, 2022, 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: August 11, 2022

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 June 30, 2022 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: August 11, 2022

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 June 30, 2022 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: August 11, 2022

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