UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2024

OR

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

Commission File Number 001-41199

Amylyx Pharmaceuticals, Inc.

(Exact name of Registrant as specified in its Charter)

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

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

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

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES ☐ NO ☒

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. YES ☐ NO ☒

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 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 has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

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

The aggregate market value of voting and non-voting common equity held by non-affiliates of the registrant, based on the closing price of the shares of common stock on the Nasdaq Global Select Market as of June 30, 2024, was $117.6 million.

The number of shares of Registrant's Common Stock outstanding as of February 28, 2025 was 88,602,468.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant intends to file a definitive proxy statement pursuant to Regulation 14A relating to the 2025 Annual Meeting of Stockholders within 120 days of the end of the registrant's fiscal year ended December 31, 2024. Portions of such definitive proxy statement for the 2025 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K to the extent stated herein.

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

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

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

- our ability to obtain regulatory approvals of avexitide in post-bariatric hypoglycemia, or PBH, or any other indications, or AMX0035 in Wolfram syndrome, progressive supranuclear palsy, or PSP, or any other indications, or any other current or future product candidates;
- the timing, progress and results of our research and development activities, preclinical studies and clinical trials, including the anticipated Phase 3 clinical program for avexitide in PBH, known as the LUCIDITY trial, our Phase 2b/3 global clinical trial of AMX0035 for the treatment of PSP known as the ORION trial, our Phase I clinical trial of AMX0114 for the treatment of amyotrophic lateral sclerosis, or ALS, known as the LUMINA trial, as well as any other development efforts, preclinical studies and clinical trials for our current and any future product candidates;
- our ability to successfully commercialize and market our product candidates, if approved, and the timing of any commercialization and marketing efforts;
- our ability to contract with third-party suppliers, manufacturers and other service providers and their ability to perform adequately and to produce sufficient quantities of clinical and, if needed, commercial supplies;
- the market size, opportunity, demand and growth potential for our current and any future product candidates, if approved;
- our ability to build and maintain our own sales and marketing capabilities, or seek collaborative partners, to commercialize our current and any future product candidates, if approved;
- our ability to obtain funding for our operations;
- our ability to retain the continued service of our key executives and to identify, hire and retain additional qualified professionals;
- our ability to successfully complete our ongoing or planned clinical trials of avexitide, AMX0035 and AMX0114 and to advance any other current or future product candidates into, and successfully complete, preclinical studies and clinical trials;
- our ability to successfully recruit and enroll suitable patients in our clinical trials;
- the timing or likelihood of the accomplishment of various scientific, clinical, regulatory filings and approvals and other product development objectives;
- the rate and degree of market acceptance of avexitide, AMX0035, and any other current or future product candidates, if approved, by physicians, patients, third-party payors and others in the medical community;
- the implementation of our business model and strategic plans for our business, products, product candidates and technology;
- our ability to identify, evaluate, in-license and develop additional products or product candidates to complement our existing pipeline and our ability to successfully incorporate acquired assets into our existing pipeline;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our products, product candidates and technology;
- developments relating to our competitors and our industry, including any regulatory developments;
- our estimates regarding expenses, potential future revenue, capital requirements, cash runway and future needs for additional financing;

•fluctuations of our quarterly and annual operating results and the related effects on our stock price;

•the effect of global financial and economic conditions and geopolitical events, including heightened and fluctuating interest rates and inflation, foreign exchange fluctuations, the risk of economic slowdown or recession in the United States, instability in the banking system, overall market volatility in the United States, including as a result of, among other factors, the ongoing war between Russia and Ukraine, the Israel-Hamas conflict and conflict in the Middle East, including political unrest, or similar events, on our business; and

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

Any forward-looking statements in this Annual 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 I, Item 1A, "Risk Factors" and elsewhere in this 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 Annual 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 Annual Report or included in our other public disclosures or our other periodic reports or other documents or filings filed with or furnished to the Securities and Exchange Commission, or the SEC, could materially and adversely affect our business, prospects, financial condition and results of operations. Except as required by law, we do not undertake or plan to update or revise any such forward-looking statements to reflect actual results, changes in plans, assumptions, estimates or projections or other circumstances affecting such forward-looking statements occurring after the date of this Annual 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 Annual Report that modify or impact any of the forward-looking statements contained in this Annual Report will be deemed to modify or supersede such statements in this Annual 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 Annual 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

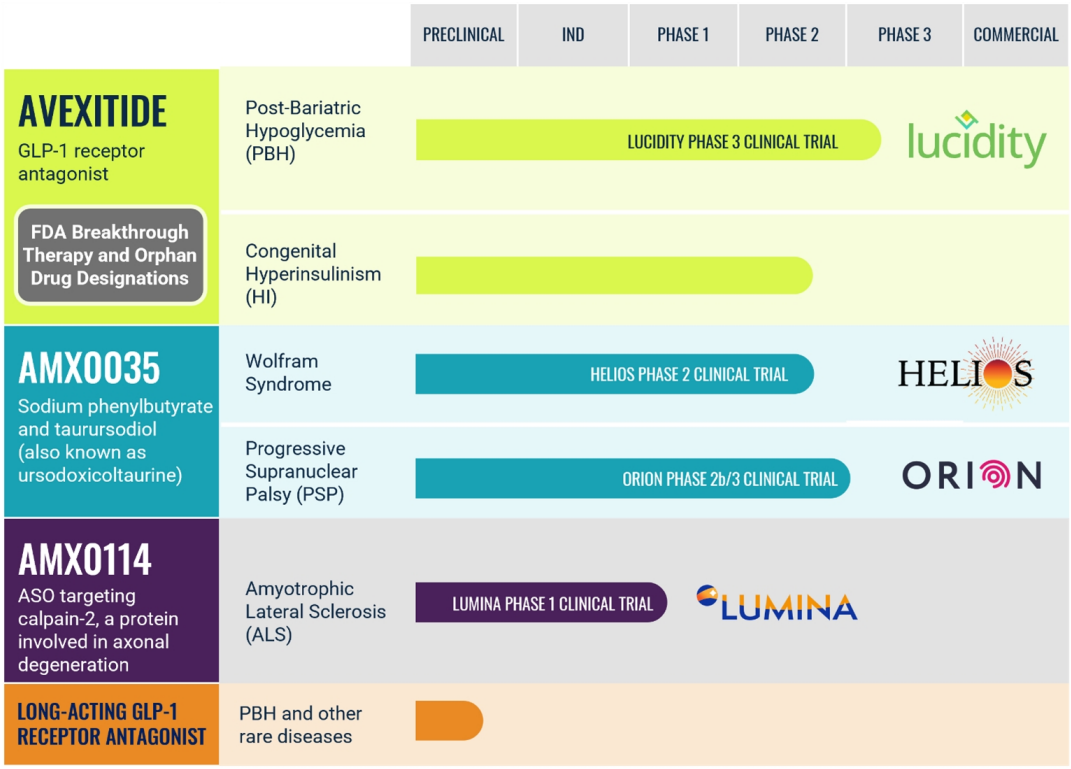
Solely for convenience, our trademarks and trade names in this report are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that we will not assert, to the fullest extent under applicable law, our rights thereto.

PART I

Item 1. Business.

Overview

Amylyx Pharmaceuticals, Inc. (also referred to as Amylyx, we, our or us) is a clinical-stage pharmaceutical company with a mission to develop and advance novel therapies for communities with high unmet medical needs. We have preclinical and clinical development programs underway in neurodegenerative diseases and endocrine conditions. We are advancing a pipeline in which we have matched investigational therapies with diseases for which we believe these therapies can make the greatest impact, based on well-defined mechanistic rationales, clear clinical outcomes and biomarkers, and rigorous preclinical data, agnostic of modality. Our current pipeline is represented in the table below.



Our lead investigational asset is avexitide, an investigational, first-in-class glucagon-like peptide-1, or GLP-1 receptor antagonist. Avexitide has been evaluated as a treatment for PBH and congenital HI, two indications characterized by hyperinsulinemic hypoglycemia. The U.S. Food and Drug Administration, or the FDA, has granted avexitide Breakthrough Therapy Designation for both post-bariatric hypoglycemia, or PBH and congenital hyperinsulinism, or HI, Rare Pediatric Disease Designation in congenital HI, and Orphan Drug Designation for the treatment of hyperinsulinemic hypoglycemia.

In PBH, an indication with no currently approved treatment options impacting approximately 160,000 people in the U.S., excessive GLP-1 can lead to the hypersecretion of insulin and subsequent persistent, recurrent, and debilitating hypoglycemic events, including autonomic and neuroglycopenic symptoms if left unaddressed. Avexitide is designed to bind to the GLP-1 receptor on pancreatic islet beta cells, inhibiting the effect of GLP-1 to mitigate hypoglycemia by decreasing insulin secretion and stabilizing blood glucose levels.

In February 2025, we started recruiting for the pivotal Phase 3 LUCIDITY clinical trial for avexitide in PBH. LUCIDITY (NCT06747468) is a multicenter, randomized, double-blind, placebo-controlled, 16-week clinical trial evaluating the efficacy and safety of avexitide in participants with PBH following Roux-en-Y gastric bypass, or RYGB surgery. The Phase 3 trial is being conducted at approximately 20 sites in the U.S. Approximately 75 participants will be randomized 3:2 to receive either 90 mg of avexitide subcutaneously once daily or placebo. Dosing is expected to begin in March or April of 2025. Participants who complete the 16-week double-blind period of the ongoing study will be eligible to enter an open-label extension, or OLE period with a duration of 32 weeks. The primary efficacy objective of LUCIDITY is to evaluate the FDA-agreed primary endpoint of reduction in the composite of Level 2 and Level 3 hypoglycemic events through Week 16. Safety and tolerability will also be evaluated. Completion of recruitment is expected in 2025, with topline data anticipated in the first half of 2026 and, if approved, commercial launch anticipated in 2027.

LUCIDITY was informed by data from five clinical trials of avexitide in people with PBH showing consistent, dose-dependent effects across studies. The five clinical trials include a Phase 1 trial, a single ascending dose trial, a multiple ascending dose trial, and two Phase 2 trials:

- In the Phase 2 (PREVENT), 28-day, randomized, placebo-controlled crossover trial (N=18), results showed a significant reduction in rates of Level 2 and 3 hypoglycemic events in participants with PBH after RYGB surgery following treatment with 30 mg twice daily and 60 mg once daily of avexitide compared with placebo. PREVENT's primary endpoint was met with statistical significance, showing both avexitide dosing regimens improved the lowest glucose level (nadir) after a meal as measured during formal mixed meal tolerance testing, or MMTT. Mean plasma glucose nadir was increased by 21% (p=0.001) and 26% (p=0.0002) following avexitide 30 mg twice daily and 60 mg once daily dosing, respectively, compared to placebo. Avexitide was generally well tolerated. The most common adverse events, or AEs, were injection site bruising, headache, and nausea; these occurred more often with placebo than either avexitide dose. No participants withdrew due to AEs.
- In the Phase 2b, 28-day, open-label, investigator-initiated, crossover trial (N=16), 90 mg once daily and 45 mg twice daily of avexitide met its primary endpoint and significantly reduced rates of hypoglycemic events in participants following RYGB surgery and other upper-gastrointestinal surgeries. Participants in the Phase 2b trial receiving 90 mg once daily of avexitide, the dose Amylyx is evaluating in LUCIDITY, saw a statistically significant 53% reduction in Level 2 hypoglycemic events (p=0.004) and a statistically significant 66% reduction in Level 3 hypoglycemic events (p=0.0003). There were no reported serious AEs, and AEs were mostly mild to moderate and resolved without medical treatment. The most common AEs included diarrhea, headache, bloating, and injection site reaction/bruising. No participant withdrew due to AEs. In the Phase 2b trial, 90 mg once daily of avexitide has also demonstrated a favorable pharmacokinetic profile maintaining exposure in the therapeutic range through 24 hours, supporting once daily dosing.

Avexitide was generally well tolerated, with a favorable safety profile replicated across five clinical trials in people with PBH. In addition, avexitide demonstrated a clear GLP-1 antagonist pharmacodynamic effect, including lowering insulin and raising the glucose nadir, in healthy volunteers.

In congenital HI, we are actively engaging in discussions with the broader congenital HI community to develop a path forward.

In December 2024, we announced a collaboration with Gubra A/S for the development of a potential novel long-acting GLP-1 receptor antagonist. As part of this collaboration, we anticipate identifying a lead development candidate to enter Investigational New Drug, or IND-enabling studies.

In addition to avexitide, we are advancing AMX0035, an oral, fixed-dose combination of sodium phenylbutyrate and taurursodiol in Wolfram syndrome and PSP, and AMX0114 in amyotrophic lateral sclerosis, or ALS.

AMX0035 is designed to mitigate neurodegeneration by targeting endoplasmic reticulum, or ER stress and mitochondrial dysfunction, two cellular processes central to neuronal cell death and neurodegeneration. We are investigating AMX0035 in neurodegenerative diseases and endocrine conditions where ER stress and mitochondrial dysfunction are implicated, including Wolfram syndrome and PSP.

Wolfram syndrome is a rare, monogenic neurodegenerative disease that progressively impacts multiple organs and systems. Wolfram syndrome is characterized by childhood-onset diabetes mellitus, optic nerve atrophy, and neurodegeneration. Common manifestations of Wolfram syndrome include diabetes mellitus and diabetes insipidus, gradual vision loss leading to blindness, hearing loss, neurogenic bladder, difficulties with balance and coordination, and difficulty

breathing that can lead to respiratory failure. There are currently no approved therapies for the approximately 3,000 people in the U.S., and more around the world, living with Wolfram syndrome.

The majority of people with Wolfram syndrome carry mutations in the WFS1 gene, which encodes a protein called wolframin that spans the membrane of the ER. Loss of wolframin function leads to ER stress and impaired mitochondrial dynamics, which lead to multi-organ cell dysfunction and death – starting with beta cells in the pancreas, then neurons in the visual system, auditory system, and throughout the body. Because of the clear link between WFS1 mutations and ER stress, Wolfram syndrome is considered a prototypical ER stress disorder. AMX0035 is hypothesized to mitigate cell death in Wolfram syndrome by reducing ER stress and mitochondrial dysfunction. In preclinical models, treatment with AMX0035 improved WFS1 protein expression, increased insulin secretion, and inhibited beta cell death in cells derived from people with Wolfram syndrome. AMX0035 also prevented cell death in neuronal cells derived from people with Wolfram syndrome and significantly delayed progression of the diabetes phenotype in a WFS1-knock-out preclinical model.

In October 2024, we announced positive topline data from the Phase 2 open-label HELIOS clinical trial of AMX0035 in 12 adults living with Wolfram syndrome. HELIOS (NCT05676034) is a single-site, single-arm, open-label, proof of biology, Phase 2 trial designed to study the effect of AMX0035 on safety and tolerability, and various measures of endocrinological, neurological, and ophthalmologic function in adult participants living with Wolfram syndrome. HELIOS showed improvement in pancreatic beta cell function, as measured by C-peptide response after 24 weeks of treatment with AMX0035, the study's primary efficacy endpoint, in contrast to the expected decrease in pancreatic function with disease progression. Similar overall improvements or stabilization were observed across all secondary endpoints, including hemoglobin A1c (HbA1c), time in target glucose range assessed by continuous glucose monitoring, and visual acuity. In addition, longer-term data for all participants who completed Week 36 (n=10) and Week 48 (n=6) assessments showed sustained improvement over time. The safety profile of AMX0035 in HELIOS was consistent with prior safety data from the study of AMX0035 in ALS. All AEs were mild or moderate, and there were no serious AEs related to AMX0035 treatment. We plan to share Week 48 data from the ongoing HELIOS trial of AMX0035 in Wolfram syndrome in 2025. Data from participants at Week 48 and regulatory interactions will inform the design of a Phase 3 trial of AMX0035 in Wolfram syndrome.

PSP is a sporadic, rare, fatal neurodegenerative disease that can affect movement, gait, balance, cognition, eye movements, swallowing, and speech. People living with PSP have a life expectancy of six to eight years after symptom onset. PSP affects approximately seven in 100,000 people worldwide, and there are currently no disease-modifying therapies approved for the treatment of PSP. Similar to other neurodegenerative diseases, the pathophysiologic changes underlying PSP are likely multifactorial, including genetic mutations, ER stress, mitochondrial dysfunction, and neuroinflammation. PSP is considered a tauopathy based on the strong genetic link between tau variants and disease development and the presence of abnormal tau protein deposits in the brain. Biomarker data from a Phase 2 trial of AMX0035 in Alzheimer's disease demonstrated a significant reduction in tau.

In December 2023, we initiated the Phase 2b/3 ORION (NCT06122662) clinical trial, a global, randomized, double-blind, placebo-controlled trial of AMX0035 for the treatment of PSP. ORION is designed to assess the efficacy and safety of AMX0035 compared to placebo. The primary objective of the ORION trial is to assess the impact of AMX0035 compared to placebo on disease progression rate as measured by the Progressive Supranuclear Palsy Rating Scale, or PSPRS, an established and validated endpoint in PSP clinical trials. Safety and tolerability will also be evaluated. Data from an unblinded interim analysis of the Phase 2b portion of the Phase 2b/3 ORION trial of AMX0035 in PSP is anticipated in the third quarter of 2025, which will be used to inform a go/no-go decision on the Phase 3 portion of the trial.

AMX0114 is an investigational antisense oligonucleotide, or ASO, targeting calpain-2, or *CAPN2*. Decades of scientific literature and published data demonstrate that *CAPN2*, a protein involved in neurofilament biology, plays an essential role in axonal degeneration, which is a critical effector in the progression of various neurodegenerative diseases including ALS. ALS is a relentlessly progressive and fatal neurodegenerative disorder caused by motor neuron death in the brain and spinal cord. Motor neuron loss in ALS leads to deteriorating muscle function, the inability to move and speak, respiratory paralysis, and, eventually, death. ALS is defined as a rare disease, but it affects as many as 30,000 adults in the U.S. and 3,000 in Canada. The most common form of the disease is sporadic ALS, with more than 90% of people with ALS showing no clear family history.

In preclinical studies, treatment with AMX0114 resulted in potent, dose-dependent, and durable reduction in *CAPN2* mRNA and calpain-2 protein levels in disease-relevant cell models of axonal degeneration. This translated to improved neuronal survival and reductions in extracellular neurofilament light chain, or NfL levels, a broadly researched biomarker for

axonal degeneration in ALS, across multiple disease models and paradigms of neuronal injury. AMX0114 was generally well tolerated in in vivo preclinical safety studies.

In February 2025, we started recruiting for the Phase 1 LUMINA clinical trial (NCT06665165), a multicenter, randomized, placebo-controlled, multiple ascending dose trial designed to evaluate the safety and biological activity of AMX0114. LUMINA will also assess ALS biomarkers, including change from baseline in NfL levels. Dosing is expected to begin in March or April of 2025. We are also working to open U.S. sites for screening, enrollment, and dosing. Approximately 48 participants will be randomized 3:1 to receive AMX0114 or placebo by intrathecal administration once every four weeks, for up to four doses. Early cohort data from LUMINA are expected in 2025.

Competition

The biotechnology and pharmaceutical industries are characterized by rapid technological advancement, significant competition and an emphasis on proprietary products. We face potential competition from many different sources, including major and specialty pharmaceutical and biotechnology companies, academic research institutions, governmental agencies, compounding pharmacies and public and private research institutions. Any product candidates that we successfully develop and commercialize may compete with current therapies and new therapies that may become available in the future. We believe that the key competitive factors affecting the success of any of our product candidates will include efficacy, safety profile, dosing, cost, effectiveness of promotional support and intellectual property protection.

Many of our competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, preclinical testing, clinical trials, manufacturing, and marketing than we do. Future collaborations and mergers and acquisitions may result in further resource concentration among a smaller number of competitors. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market or make our development more complicated. These competitors may also vie for a similar pool of qualified scientific and management talent, sites and patient populations for clinical trials, as well as for technologies complementary to, or necessary for, our programs.

Supply and Manufacturing

We rely, and expect to continue to rely for the foreseeable future, on third-party contract manufacturing organizations, or CMOs, for the production of avexitide, AMX0035 and AMX0114 in compliance with current Good Manufacturing Process, or cGMP, requirements, for use in clinical trials under the guidance of members of our organization. We have long-term, single-source supply agreements in place for our active pharmaceutical ingredients, and single-source arrangements for the manufacturing and packaging of bulk drug at established CMOs for clinical trials and other potential needs.

We have built a team of pharmaceutical industry technical operations leaders. This team has significant technical, manufacturing, analytical, quality, regulatory, including cGMP, and project management experience to oversee our third-party manufacturers and maintain quality and regulatory compliance.

We also have a Quality Management System consistent with a regulated industry that outlines Standard Operating Policies and Procedures that govern the oversight of our CMOs.

Intellectual Property

Our commercial success depends in part on our ability to obtain intellectual property that protects avexitide and its uses, AMX0035 and its uses, AMX0114 and its uses, and any future product candidates. We seek to protect and enhance proprietary technology, inventions and improvements that are commercially important to the development of our business by seeking, maintaining and defending U.S. and foreign patent rights.

We are actively building our intellectual property portfolio in our therapeutic areas, including around avexitide, AMX0035, and AMX0114. As of December 31, 2024, our patent estate included 20 issued U.S. patents, 25 pending U.S. patent applications, 211 granted foreign patents, and 109 pending foreign patent applications.

Avexitide

We acquired a patent portfolio directed to avexitide from Eiger Pharmaceuticals, Inc., or Eiger, in June 2024. The portfolio includes one in-licensed patent family from University of Pennsylvania/Children's Hospital of Philadelphia, two in-licensed patent families from Stanford University, one co-owned with and in-licensed patent family from Stanford University, one patent family co-owned by us and Stanford University, and two patent families that are solely owned by us.

The in-licensed patent family from University of Pennsylvania/Children's Hospital of Philadelphia relates to compositions and methods for treating congenital and neonatal hyperinsulinism and post-prandial hypoglycemia. This family includes 4 issued U.S. patents, and 58 issued foreign patents. We also have patent applications pending in this family in the U.S. and EU. The issued patents and others that issue from this family may first begin to expire as early as January 8, 2028, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely.

The two in-licensed patent families from Stanford University relate to treatment of hyperinsulinemic hypoglycemia with GLP-1 antagonist exendin(9-39). There are 7 issued U.S. patents and 81 issued foreign patents in these two families. We also have patent applications pending in these two families in the U.S., EU, and other jurisdictions. The issued patents and others that issue from these families may first begin to expire as early as May 23, 2026, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely.

The co-owned with, and in-licensed patent family from, Stanford University relates to liquid pharmaceutical formulations of exendin(9-39). There are 2 issued U.S. patents and 5 issued foreign patents in this family. We also have patent applications pending in this family in the U.S., EU, and other jurisdictions. The issued patents and others that issue from this family may first begin to expire as early as November 21, 2037, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely.

Also included are the two patent families relating to avexitide that are solely owned by us. One family relates to the treatment of hyperinsulinemic hypoglycemia with exendin(9-39). There are currently no issued patents in this family. We have patent applications pending in this family in the U.S., EU, and other jurisdictions. Any patents to issue from this family may first begin to expire as early as October 15, 2039, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely. Another family relates to the treatment of congenital HI with exendin(9-39). There are currently no issued patents in this family. We have patent applications pending in this family in the U.S., EU, and other jurisdictions. Any patents to issue from this family may first begin to expire as early as June 21, 2042, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely.

The remaining patent family, which is co-owned by us and Stanford University, relates to methods of improving nutrition in subjects, including individuals who have undergone gastrointestinal surgery, by avexitide therapy. We have a Patent Cooperation Treaty, or PCT application pending in this family. Although no patents have yet issued from this family, we expect the term on patents issuing from this family to extend until at least April 2044, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely.

AMX0035

Our patent portfolio around AMX0035 includes ten patent families. In those ten families, we currently own a total of 164 issued patents and pending patent applications. Currently, our patent portfolio around AMX0035 includes seven issued U.S. patents and 67 issued foreign patents. We also have pending applications in the U.S., EU and other jurisdictions. Our issued patents and pending applications cover the relative amounts of a phenylbutyrate compound and a bile acid (such as TUDCA) and some of our issued and pending claims cover the specific ratio of those two drugs.

The patent families around AMX0035 include those that relate to compositions of a bile acid and a phenylbutyrate compound (including TURSO and 4-PBA) and methods of treating neurodegenerative disease, and its associated causes at a cellular level, using those compositions; specific compositions of a phenylbutyrate compound and a bile acid (including TURSO and 4-PBA) and methods of manufacturing those compositions; methods of treating particular symptoms of ALS and/or reducing the associated AEs with combinations of a phenylbutyrate compound and a bile acid (including TURSO and 4-PBA); methods of treating Alzheimer's disease and other tauopathies (including progressive supranuclear palsy) with

combinations of a phenylbutyrate compound and a bile acid (including sodium phenylbutyrate and TURSO); methods of co-administering other therapeutic drugs with combinations of a phenylbutyrate compound and a bile acid (including sodium phenylbutyrate and TURSO); pharmacokinetic characteristics following the administration of TURSO and sodium phenylbutyrate; methods of reducing the level of certain biomarkers in ALS patients with combinations of sodium phenylbutyrate and TURSO; and methods of treating Wolfram Syndrome with combinations of sodium phenylbutyrate and TURSO. The issued patents and others that issue from our earliest in time patent family around AMX0035 may first begin to expire as early as December 2033, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely. Although no patents have issued from the latest patent family around AMX0035, we expect the term of patents issued from that family to extend until at least September 2043, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely.

AMX0114

Our patent portfolio around AMX0114 includes 1 patent family. This patent family relates to oligonucleotides targeting the Calpain-2 mRNA transcript. We have patent applications pending in this family in the U.S., EU, and other jurisdictions. Although no patents have yet issued from this family, we expect the term on patents issuing from this family to extend until at least May 9, 2043, not accounting for any patent term adjustment or extensions or terminal disclaimers, and assuming that all applicable annuity and/or maintenance fees are paid timely.

We cannot be sure that patents will be granted with respect to any of our pending patent applications nor with respect to any patent applications that may be filed by us in the future. Further, we cannot be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products. Finally, we cannot be sure that our granted patents, and any future patents granted to us, will be found valid and/or enforceable following a litigation or administrative procedure.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing of the first non-provisional application to which priority is claimed. In the U.S., patent term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. In the U.S., the term of a patent that covers an FDA-approved drug may also be eligible for a patent term extension of up to five years under the Hatch-Waxman Act, which is designed to compensate for the patent term lost during the FDA regulatory review process. The length of the patent term extension is calculated based on the length of time it takes for regulatory review. A patent term extension under the Hatch-Waxman Act cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Moreover, a patent can only be extended once, and thus, if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug. Following the approval of RELYVRIO in the U.S., we applied for patent term extensions for certain of our issued U.S. patents covering our product candidates and/or their methods of use.

We also rely on trademarks, trade secrets, know-how, continuing technological innovation, confidentiality agreements, and invention assignment agreements to develop and maintain our proprietary position. The confidentiality agreements are designed to protect our proprietary information and the invention assignment agreements are designed to grant us ownership of technologies that are developed for us by our employees, consultants, or other third parties. We seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology, or IT, systems. While we have confidence in our agreements and security measures, either may be breached, and we may not have adequate remedies. In addition, our trade secrets may otherwise become known or independently discovered by competitors.

Our potential commercial success also depends in part on our ability to operate without infringing on the proprietary rights of others and to prevent others from infringing our proprietary rights. A comprehensive discussion on risks relating to intellectual property is provided in Item 1A of this Annual Report entitled "Risk Factors—Risks Related to Our Intellectual Property."

European Patent EP3016654, entitled "Tauroursodeoxycholic acid, or TUDCA for Use in the Treatment of Neurodegenerative Disorders," is owned by Bruschettini S.r.l. The patent relates to use of TURSO in the treatment of ALS in a mammal. An opposition has been filed to the grant of EP3016654 at the European Patent Office, or EPO, asking the EPO to

revoke EP3016654. The EPO issued a preliminary opinion on November 18, 2019 finding that at least the main claim of EP3016654 lacked novelty. Oral proceedings were held before an Opposition Division of the EPO on June 11, 2021. At the end of the oral proceedings, the Opposition Division announced the decision revoking all claims of EP3016654. A written decision has been issued; however Bruschettini has appealed the decision of the Opposition Division to the Board of Appeal. A response to Bruschettini's appeal has been filed on June 7, 2022 requesting that the appeal should be dismissed and that the decision of the Opposition Division to revoke all claims of EP3016654 be upheld. The Board of Appeal issued a summons to attend oral proceedings on May 24, 2023. The Board of Appeal overturned in oral proceedings held on June 5, 2024, the decision of the Opposition Division and maintained EP3016654 in limited form. The patent as maintained in limited form protects TUDCA for use in the treatment of ALS only. As such, EP3016654 as maintained has no relevance for PSP or Wolfram Syndrome. Bruschettini has no procedural option to broaden the claims at this point. A European divisional application is not pending in this family and cannot be filed anymore.

Government Regulation

The FDA and comparable regulatory authorities in state and local jurisdictions and in other countries, including Canada and member states of the EU impose requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs, such as those we are developing. These agencies and other federal, state and local entities regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of our product candidates.

U.S. Government Regulation of Drug Products

In the U.S., the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the U.S. generally involves the following:

•Completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;

•Submission to the FDA of an IND, which must become effective before human clinical trials may begin;

•Approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated;

•Performance of adequate and well-controlled human clinical trials in accordance with Good Clinical Practices, or GCP, requirements to establish the safety and efficacy of the proposed drug product for each indication;

•Submission to the FDA of an NDA, including payment of application user fees;

•A determination by the FDA within 60 days of its receipt of a new drug application, or an NDA to accept the marketing application for review;

•Satisfactory completion of an FDA advisory committee review, if applicable;

•Satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;

•Satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data; and

•FDA review and approval of the NDA.

Preclinical Studies

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as *in vitro* and animal studies to assess potential safety and efficacy. The conduct of preclinical studies is subject to federal regulations and requirements, including good laboratory practice regulations for safety/toxicology studies.

An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data and any available clinical data or literature and plans for clinical studies, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before human clinical trials may begin. Some preclinical testing, such as animal tests of reproductive AEs and carcinogenicity, may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to initiate.

Clinical Trials

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it is initiated at that institution. The IRB also must review and approve the informed consent form that must be provided to each clinical trial subject or his or her legal representative, and must monitor the clinical trial until completion.

Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on their www.clinicaltrials.gov website. Information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Although sponsors are obligated to disclose the results of their clinical trials after completion, disclosure of the results can be delayed in some cases for up to two years after the date of completion of the trial. Failure to timely register a covered clinical study or to submit study results as provided for in the law can give rise to civil monetary penalties and also prevent the non-compliant party from receiving future grant funds from the federal government.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase 1: The drug is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.
- Phase 2: The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- Phase 3: The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval on an NDA.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if suspected AEs occur. Written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected AEs, findings from other studies or animal or *in vitro* testing that suggest a significant risk for human

subjects and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information.

The FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether a trial may move forward at designated check points based on access to certain data from the trial.

Concurrent with clinical trials, companies usually complete additional animal studies and also must develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of product and, among other things, companies must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug does not undergo unacceptable deterioration over its shelf life.

Expanded Access to an Investigational Drug for Treatment Use

Expanded access, sometimes called "compassionate use," is the use of investigational products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational products for patients who may benefit from investigational therapies. FDA regulations allow access to investigational products under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the investigational product under a treatment protocol or treatment IND application.

There is no obligation for a sponsor to make its drug products available for expanded access; however, as required by the 21st Century Cures Act, or Cures Act, passed in 2016, a sponsor must make its expanded access policy publicly available upon the earlier of initiation of a Phase 2 or Phase 3 trial; or 15 days after the investigational drug or biologic receives fast track, breakthrough or regenerative medicine advanced therapy designation.

In addition, on May 30, 2018, the Right to Try Act was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a manufacturer to make its investigational products available to eligible patients as a result of the Right to Try Act.

NDA Submission and Marketing Approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. The FDA will initially review an NDA for completeness before it accepts it for "filing." Under the FDA's procedures, the agency has 60 days from its receipt of the NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that the application is sufficiently complete to permit substantive review. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA, for a new molecular entity to review and act on the submission, and six months from the filing date of a new molecular entity NDA with priority review. Accordingly, this review process typically takes 12 months and 8 months, respectively from the date the NDA is submitted to the FDA. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs, and the review process is often extended by FDA requests for additional information or

clarification. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective for its intended use(s), with the latter determination being made on the basis of substantial evidence. This finding can be substantiated based on two adequate and well-controlled studies, or in certain circumstances on a single, large, multicenter, adequate and well-controlled study that is very persuasive or from a single adequate and well-controlled study together with confirmatory evidence. FDA regulations and guidance also allow for greater flexibility and tolerance for uncertainty in the context of rare and fatal diseases. The FDA also assesses whether the facility in which the product is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

In addition, under the Pediatric Research Equity Act of 2003, or PREA, as amended, certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration must submit an initial Pediatric Study Plan within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before initiation of the Phase 3 or Phase 2/3 study. The initial Pediatric Study Plan must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the Pediatric Study Plan. A sponsor can submit amendments to an agreed-upon initial Pediatric Study Plan at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review.

The FDA may refer an application for a novel drug or a drug that presents difficult questions of safety or efficacy to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, which reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA also may require the submission of a Risk Evaluation and Mitigation Strategy, or REMS, if it determines that a REMS is necessary to ensure that the benefits of the drug outweigh its risks and to assure the safe use of the drug. A REMS may include one or more elements, including medication guides, physician communication plans, patient package insert and/or elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools. The FDA determines the requirement for a REMS, as well as the specific REMS provisions, on a case-by-case basis. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS. The FDA will not approve the NDA without a REMS, if required.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP requirements.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a Complete Response Letter. A Complete Response Letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for FDA to reconsider the application. If a Complete Response Letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an

approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug product intended to treat a rare disease or condition, which is generally a disease or condition that affects either (i) fewer than 200,000 individuals in the U.S., or (ii) more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making the product available in the U.S. for this type of disease or condition will be recovered from sales of the product. A company must request orphan drug designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product is entitled to orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications to market the same product for the same indication for seven years, except in certain limited circumstances. If a drug designated as an orphan drug ultimately receives marketing approval for an indication broader than what it was designated for, it may not be entitled to exclusivity. Orphan exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan drug has 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.

Expedited Development and Priority Review Programs

The FDA maintains several programs intended to facilitate and expedite development and review of new drugs to address unmet medical needs in the treatment of serious or life-threatening diseases or conditions. These programs include Fast Track Designation, Breakthrough Therapy Designation, Priority Review Designation and accelerated approval, and the purpose of these programs is to either expedite the development or review of important new drugs to get them to patients earlier than under standard FDA development and review procedures.

The FDA has a FastTrack program that is intended to expedite or facilitate the process for reviewing new drugs that meet certain criteria. Specifically, new drugs are eligible for Fast Track Designation if they are intended to treat a serious or life threatening condition and preclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast Track Designation applies to both the product and the specific indication for which it is being studied. The sponsor can request the FDA to designate the product for Fast Track status any time before receiving NDA approval, but ideally no later than the pre-NDA meeting. Fast Track Designation provides increased opportunities for sponsor interactions with the FDA during preclinical and clinical development, in addition to the potential for rolling review once a marketing application is filed, meaning that the agency may review portions of the marketing application before the sponsor submits the complete application, as well as priority review, discussed below.

Additionally, a drug may be eligible for designation as a breakthrough therapy if the product is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening condition and preliminary clinical

evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints. The benefits of Breakthrough Therapy Designation include the same benefits as Fast Track Designation, plus intensive guidance from the FDA to ensure an efficient drug development program. A product may also be eligible for priority review if it treats a serious or life-threatening condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA determines at the time that the marketing application is submitted, on a case-by-case basis, whether the proposed drug represents a significant improvement in treatment, prevention or diagnosis of disease when compared with other available therapies. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review in an effort to facilitate the review and to shorten the FDA's goal for taking action on an NDA for a new molecular entity from ten months to six months from the date of filing.

A product may also be eligible for accelerated approval if it treats a serious or life-threatening disease or condition, generally provides a meaningful advantage over available therapies and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. As a condition of accelerated approval, the FDA may require that a sponsor perform adequate and well-controlled post-marketing confirmatory trials with due diligence and, under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA is now permitted to require, as appropriate, that such trials be underway prior to approval or within a specific time period after the date accelerated approval is granted. Under FDORA, the FDA also has increased authority for expedited procedures to withdraw approval of a drug or indication approved under accelerated approval if, for example, the confirmatory trial fails to verify the predicted clinical benefit of the product. In addition, the FDA generally requires, unless otherwise informed by the agency, pre-approval of promotional materials for products considered for accelerated approval, which could adversely impact the timing of the commercial launch of the product.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Fast Track Designation, Breakthrough Therapy designation and Priority Review designation do not change the standards for approval, but may expedite the development or review process. Drugs granted accelerated approval also must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

U.S. Non-Patent Exclusivity

Data exclusivity provisions under the FDCA can delay the submission or the approval of certain follow-on applications. The FDCA provides a five-year period of data exclusivity within the U.S. to the first applicant to gain approval of an NDA for a new chemical entity, or NCE. A drug is an NCE if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an ANDA for a generic version of the drug or a 505(b)(2) NDA for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, such a follow-on application may be submitted after four years if it contains a certification of patent invalidity or non-infringement.

The FDA has previously taken the position that NCE exclusivity is not available for fixed-dose combination products if one of the active moieties in the combination product had been previously approved in a drug product. In October 2014, however, the FDA reversed that position when it issued final guidance stating that an application for a fixed-dose combination product will be eligible for 5-year NCE exclusivity if it contains a drug substance with a single, new active moiety, even if the fixed-combination also contains a drug substance with a previously approved active moiety.

The FDCA also provides three years of market exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity period covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving follow-on applications that do not reference the protected clinical data. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of regulatory market exclusivity in the U.S. Pediatric exclusivity, if granted, adds six months to existing regulatory exclusivity periods for all formulations, dosage forms, and indications of the active

moiety and patent terms. This six-month exclusivity may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued "Written Request" for such a trial, provided that at the time pediatric exclusivity is granted there is not less than nine months of term remaining.

Post-approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There are continuing, annual user fee requirements for any marketed products.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

FDA regulations require that products be manufactured in specific facilities and in accordance with cGMP regulations which require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs, including those supply products, ingredients and components thereof, are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. In addition, the Drug Supply Chain Security Act, or DSCSA, was enacted in 2013 with the aim of building an electronic system to identify and trace certain prescription drugs and biologics distributed in the United States. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use.

Once an approval of a drug is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market, such as if, based on new evidence of clinical experience not contained in the application or not available to the FDA until after the application was approved, there is a lack of substantial evidence that the approved product will have the effect it is purported or represented to have under the conditions of use prescribed, recommended, or suggested in its labeling. Sponsors may also voluntarily withdrawal their approved products from the market for similar reasons. Later discovery of previously unknown problems with a product, including AEs of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Other potential consequences include, among other things:

- Restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- Fines, warning letters or holds on post-approval clinical trials;
- Refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or withdrawal of product approvals;
- Product seizure or detention, or refusal to permit the import or export of products; and
- Injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted by a manufacturer and any third parties acting on behalf of a manufacturer only for the approved indications and in a manner consistent with the approved label for the product. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

From time to time, legislation is drafted, introduced, passed in Congress and signed into law that could significantly change the statutory provisions governing the approval, manufacturing, and marketing of products regulated by the FDA. In

addition to new legislation, FDA regulations, guidance, and policies are often revised or reinterpreted by the agency in ways that may significantly affect the manner in which pharmaceutical products are regulated and marketed.

Other U.S. Healthcare Laws

Healthcare providers, physicians, and third-party payors will play a primary role in the recommendation and prescription of drug products for which we obtain marketing approval. Arrangements with third-party payors, healthcare providers and physicians, as well as patients and other third parties, in connection with the clinical research, sales, marketing and promotion of products, once approved, and related activities, may expose a pharmaceutical manufacturer to broadly applicable fraud and abuse and other healthcare laws and regulations. In the U.S., these laws include, without limitation, state and federal anti-kickback, false claims, physician transparency, and patient data privacy and security laws and regulations, including but not limited to those described below:

- the Anti-Kickback Statute, or AKS, which makes it illegal for any person or entity, including a prescription drug manufacturer (or a party acting on its behalf) to knowingly and willfully solicit, receive, offer or pay any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, that is intended to induce or reward, referrals including the purchase, recommendation, order or prescription of a particular drug for which payment may be made under a federal healthcare program, such as the Medicare and Medicaid programs. The AKS has been interpreted to apply to arrangements between therapeutic product manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Further, courts have found that if "one purpose" of remuneration is to induce referrals, the AKS is violated. In addition, the government may assert that a claim including items or services resulting from a violation of the AKS constitutes a false or fraudulent claim for purposes of the federal False Claims Act, or FCA;

- the federal civil and criminal false claims laws, including the FCA, which can be enforced by private citizens through "qui tam" or "whistleblower" actions, and civil monetary penalty laws, which impose criminal and civil penalties against individuals or entities for, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal health care programs that are false or fraudulent; knowingly making or causing a false statement material to a false or fraudulent claim or an obligation to pay or transmit money or property to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing such an obligation. Manufacturers can be held liable under the federal False Claims Act even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims. Pharmaceutical and other healthcare companies have been, and continue to be, prosecuted under these laws, among other things, for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product and for causing false claims to be submitted because of the companies' marketing of the product for unapproved, off-label, and thus generally non-reimbursable, uses. Similar to the AKS, a person or entity does not need to have actual knowledge of these statutes or specific intent to violate them in order to have committed a violation.

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Like the AKS, the Patient Protection and Affordable Care Act, or the ACA, amended the intent standard for certain healthcare fraud statutes under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the creation, use, receipt, maintenance or disclosure of individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorneys' fees and costs associated with pursuing federal civil actions;

•the federal Physician Payments Sunshine Act, created under the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the Centers for Medicare and Medicaid Services, or CMS, under the Open Payments Program, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), to certain non-physician providers such as physician assistants and nurse practitioners, and to teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;

•federal price reporting laws, which require manufacturers to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on approved products;

•federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and

•analogous state and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to file reports with states regarding pricing and marketing information, such as the tracking and reporting of gifts, compensations and other remuneration and items of value provided to healthcare professionals and entities; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of a pharmaceutical manufacturer's business activities could be subject to challenge under one or more of such laws. Efforts to ensure that business arrangements comply with applicable healthcare laws involve substantial costs. It is possible that governmental and enforcement authorities will conclude that a pharmaceutical manufacturer's business practices do not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against a pharmaceutical manufacturer, and it is not successful in defending itself or asserting its rights, those actions could have a significant impact on its business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, imprisonment, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, reporting obligations and oversight if we become subject to integrity and oversight agreements to resolve allegations of non-compliance, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of operations, any of which could adversely affect a pharmaceutical manufacturer's ability to operate its business and the results of operations. In addition, commercialization of any drug product outside the U.S. will also likely be subject to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

In the U.S., federal and state laws and regulations, including state data breach notification laws, state and federal health information privacy laws, and federal and state consumer protection laws, govern our collection, use, disclosure, and protection of personal information. In California, for example, the California Consumer Privacy Act, or CCPA, requires covered businesses to include certain disclosures to California consumers about how their data is collected, used and shared and provide such individuals the ability to opt-out of certain sales or transfers of their personal information to third parties. Numerous other U.S. states have passed similarly comprehensive consumer privacy laws which may vary in their scope and application, and enforcement will likely remain unpredictable for the foreseeable future. Other states have passed privacy legislation which applies specifically to consumer health data. For example, Washington's My Health My Data Act, which entered into force on March 31, 2024, expands the definition of consumer health data, affords consumers with privacy rights and creates a private right of action, which could increase the risk of, and expenses related to, litigation. While the CCPA contains an exception for certain activities involving PHI under HIPAA, we cannot yet determine the impact the CCPA or other such future laws, regulations and standards may have on our business, as these laws either do not yet apply to us or are not yet in effect. Certain state laws may be more stringent or broader in scope than others, or offer greater individual rights with respect to personal information than federal, international or other state laws with potentially conflicting requirements, which may complicate our compliance efforts. Any failure or perceived failure to comply with any of these laws could negatively impact our business, including through enforcement actions, litigation and reputational harm leading to loss of existing and future business.

Current and Future U.S. Healthcare Reform Legislation

Payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs and those methods are not always specifically adapted for new technologies such as gene therapy and therapies addressing rare diseases such as those we are developing. In both the U.S. and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, was enacted, which, among other things, subjected biologic products to potential competition by lower-cost biosimilars; increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations; subjected manufacturers to new annual fees and taxes for certain branded prescription drugs; created the Medicare Part D coverage gap discount program, in which manufacturers must agree to 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; and provided incentives to programs that increase the federal government's comparative effectiveness research.

Other legislative changes have been proposed and adopted since the ACA was enacted. For example. the Budget Control Act of 2011 and subsequent legislation, among other things, created measures for spending reductions that include aggregate reductions of Medicare payments to providers of 2% per fiscal year, which remain in effect through 2031. Also, the American Taxpayer Relief Act of 2012 further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Due to the Statutory Pay-As-You-Go Act of 2010, estimated budget deficit increases resulting from the American Rescue Plan Act of 2021, and subsequent legislation, Medicare payments to providers were further reduced starting on January 1, 2025; however, legislation has been introduced in the U.S. Congress that would reverse these payment reductions. In addition to provider payment cuts under Medicare, the American Rescue Plan Act of 2021 also eliminated the statutory Medicaid drug rebate cap, previously set at 100% of a drug's average manufacturer price, for single source innovator multiple source drugs, beginning January 1, 2024. These laws and regulations may result in additional reductions in Medicare and other healthcare funding available for healthcare providers and may otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

The Inflation Reduction Act of 2022, or IRA, includes several provisions that may impact our business to varying degrees, including provisions that reduce the out-of-pocket spending cap for Medicare Part D beneficiaries from $7,050 to $2,000 starting in 2025, thereby effectively eliminating the coverage gap; impose new manufacturer financial liability on certain drugs under Medicare Part D, allow the U.S. government to negotiate Medicare Part B and Part D price caps for certain high-cost drugs and biologics without generic or biosimilar competition; require companies to pay rebates to Medicare for certain drug prices that increase faster than inflation; and delay until January 1, 2032 the implementation of the HHS rebate rule that would have limited the fees that pharmacy benefit managers can charge. Further, under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication is for that disease or condition. If a product receives multiple orphan designations or has multiple approved indications, it may not qualify for the orphan drug exemption. The implementation of the IRA is currently subject to ongoing litigation that challenges the constitutionality of the IRA's Medicare drug price negotiation program. The effects of the IRA on our business and the healthcare industry in general is not yet known.

European Union Approval Process

The process governing approval of medicinal products in the EU generally follows the same lines as in the U.S. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application, or MAA, and granting of a marketing authorization by these authorities before the product can be marketed and sold in the EU.

Clinical Trial Approval

In April 2014, the EU adopted the new Clinical Trials Regulation (EU) No 536/2014, or Clinical Trials Regulation, which replaced the Clinical Trials Directive 2001/20/EC on January 31, 2022. All clinical trials in the EU must now be conducted in accordance with the Clinical Trials Regulation. The legislation aims at simplifying and streamlining the approval of clinical trials in the EU; for example, the Clinical Trials Regulation provides for a streamlined application procedure via a single-entry point, rules on the protection of subjects and informed consent, transparency requirements, and strictly defined deadlines for the assessment of clinical trial applications.

PRIME Designation in the EU

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIority MEdicines, or PRIME, scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation where the marketing authorization application will be made through the centralized procedure. Eligible products must target conditions for which there is an unmet medical need (there is no satisfactory method of diagnosis, prevention or treatment in the EU or, if there is, the new medicine will bring a major therapeutic advantage) and they must demonstrate the potential to address the unmet medical need by introducing new methods of therapy or improving existing ones. Products from small- and medium-sized enterprises, or SMEs, may qualify for earlier entry into the PRIME scheme than larger companies. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and accelerated MAA assessment once a dossier has been submitted. Importantly, a dedicated contact and rapporteur from the EMA's CHMP, or Committee for Advanced Therapies, are appointed early in PRIME scheme facilitating increased understanding of the product at EMA's Committee level. A kick-off meeting initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies.

Fixed-Dose Combination Guideline

As with the FDA, the EMA has also issued guidelines to address review and approval of fixed-dose combination products. This EMA's Guideline on clinical development of fixed combination medicinal products came into force on October 1, 2017. The basic scientific requirements for any fixed combination medicinal product are justification of the pharmacological and medical rationale for the combination, and establishment of the evidence base for the relevant contribution of all active substances to the desired therapeutic effect (efficacy and/or safety) and a positive benefit-risk for the combination in the targeted indication. For products that involve initial combination of two active ingredients, the EMA has indicated that the design of clinical efficacy/safety studies to support a fixed combination medicinal product application for initial treatment will depend on its rationale, specifically to achieve superior efficacy or improved safety compared to use of the single active substances. In situations when it has been established that monotherapy will not be adequate, appropriate or ethical to reach the desired therapeutic effect, initial use of combination therapy should be easily justified (e.g., HIV).

Marketing Authorization

To obtain a marketing authorization for a product in the European Economic Area (i.e., the EU as well as Iceland, Liechtenstein and Norway), or EEA, an applicant must submit an MAA either under a centralized procedure administered by the EMA, or one of the procedures administered by competent authorities in the EU Member States (decentralized procedure, national procedure or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the EEA. Regulation (EC) No 1901/2006 provides that prior to obtaining a marketing authorization in the EU, applicants have to demonstrate compliance with all measures included in an EMA-approved Paediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted (1) a product-specific waiver, (2) a class waiver or (3) a deferral for one or more of the measures included in the PIP (for example, when this data is not needed or appropriate because the product is likely to be ineffective or unsafe in children, the disease or condition for which the product is intended occurs only in adult populations, or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients). Products that are granted a marketing authorization with the results of the pediatric clinical trials conducted in accordance with the PIP (even where such results are negative) are eligible for six months' supplementary protection certificate, or SPC, extension (provided an application for such extension is made at the same time as filing the SPC application for the product, or at any point up to 2 years before the SPC expires).

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid across the EEA. Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy medicinal products (*i.e.* gene therapy, somatic-cell therapy and tissue-engineered medicinal products)and products with a new active substance indicated for the treatment of certain diseases, including HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of public health, the centralized procedure is optional. The centralized procedure may at the request of the applicant also be used in certain other cases. We anticipate that the centralized procedure will be mandatory for the product candidates we are developing.

Under the centralized procedure, the CHMP is responsible for conducting the initial assessment of a product and for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Clock stops may extend the timeframe of evaluation of an MAA considerably beyond 210 days. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. If the CHMP accepts such request, the time limit of 210 days will be reduced to 150 days (excluding clock stops) but it is possible that the CHMP can revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment. At the end of this period, the CHMP provides a scientific opinion on whether or not a marketing authorization should be granted in relation to a medicinal product. Where the CHMP gives a positive opinion, the EMA provides the opinion together with supporting documentation to the European Commission, who makes the final decision to grant a marketing authorization, which is issued within 67 days of receipt of the EMA's recommendation.

The European Commission may grant a so-called "marketing authorization under exceptional circumstances". Such authorization is intended for products for which the applicant can demonstrate that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use, because either (i) the indications for which the product in question is intended are encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence; (ii) in the present state of scientific knowledge, comprehensive information cannot be provided; or (iii) it would be contrary to generally accepted principles of medical ethics to collect such information. Consequently, marketing authorization under exceptional circumstances may be granted subject to certain specific obligations, which may include the following:

- the applicant must complete an identified program of studies within a time period specified by the competent authority, the results of which form the basis of a reassessment of the benefit/risk profile;
- the medicinal product in question may be supplied on medical prescription only and may in certain cases be administered only under strict medical supervision, possibly in a hospital and in the case of a radiopharmaceutical, by an authorized person; and
- the package leaflet and any medical information must draw the attention of the medical practitioner to the fact that the particulars available concerning the medicinal product in question are as yet inadequate in certain specified respects.

A marketing authorization under exceptional circumstances is subject to annual review to reassess the risk-benefit balance in an annual reassessment procedure. Continuation of the authorization is linked to the annual reassessment and a negative assessment could potentially result in the marketing authorization being suspended or revoked. The renewal of a marketing authorization of a medicinal product under exceptional circumstances, however, follows the same rules as a "normal" marketing authorization. Thus, a marketing authorization under exceptional circumstances is granted for an initial five years, after which the authorization will become valid indefinitely, unless the EMA decides that safety grounds merit one additional five-year renewal.

Unlike the centralized authorization procedure, the decentralized marketing authorization procedure requires a separate application to, and leads to separate approval by, the competent authorities of each EU Member State in which the product is to be marketed. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States who, within 90 days of receipt, must decide whether to approve the assessment report and related materials. If a concerned EU Member State cannot approve the assessment report and related materials due to

concerns relating to a potential serious risk to public health, disputed elements may be referred to the European Commission, whose decision is binding on all EU Member States.

The mutual recognition procedure similarly is based on the acceptance by the competent authorities of the EU Member States of the marketing authorization of a medicinal product by the competent authorities of other EU Member States. The holder of a national marketing authorization may submit an application to the competent authority of an EU Member State requesting that this authority recognize the marketing authorization delivered by the competent authority of another EU Member State.

Conditional Marketing Authorization

The European Commission may also grant a so-called "conditional marketing authorization" prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional marketing authorizations may be granted for product candidates intended for treating, preventing or diagnosing seriously debilitating or life-threatening diseases (including medicines designated as orphan medicinal products) or in a public health emergency, if (i) the risk-benefit balance of the product candidate is positive, (ii) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data post-authorization, (iii) the product fulfills an unmet medical need and (iv) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization. A conditional marketing authorization can be converted into a standard centralized marketing authorization (no longer subject to specific obligations) once the marketing authorization holder fulfils the obligations imposed and the complete data confirm that the medicine's benefits continue to outweigh its risks.

Regulatory Data Protection in the EU

In the EU, innovative medicinal products approved on the basis of a complete and independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. Data exclusivity, if granted, prevents applicants for authorization of generics or biosimilars of these innovative products 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 an additional two-year period of market exclusivity, a generic or biosimilar MAA can be submitted and authorized, and the innovator's data may be referenced, but no generic or biosimilar medicinal product can be placed on the EU market until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be an innovative medical product so that the innovator gains the prescribed period of data exclusivity, another company nevertheless could also market another version of the product if such company obtained marketing authorization based on an MAA with a complete and independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Periods of Authorization and Renewals

A marketing authorization has an initial validity for five years in principle. The marketing authorization may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA (for a centrally authorized product) or by the competent authority of the relevant EU Member State (for a nationally authorized product). To this end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least nine months before the marketing authorization ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide, on justified grounds relating to pharmacovigilance, to proceed with one further five-year period of marketing authorization. Once subsequently definitively renewed, the marketing authorization shall be valid for an unlimited period. Any authorization which is not followed by the actual placing of the medicinal product on the EU market (in case of centralized procedure) or on the market of the authorizing EU Member State (for a nationally

authorized product) within three years after authorization, or if the product is removed from the market for three consecutive years, ceases to be valid (the so-called sunset clause).

Orphan Drug Designation and Exclusivity

Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a medicinal product can be designated as an orphan medicinal product by the European Commission if its sponsor can establish that: (1) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (i) such condition affects no more than five in ten thousand persons in the EU when the application is made, or (ii) without incentives it is unlikely that the marketing of the product in the EU would generate sufficient return to justify the necessary investment in its development; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the product will be of significant benefit to those affected by that condition.

Once authorized, orphan medicinal products are entitled to ten years of market exclusivity in all EU Member States and a range of other benefits during the development and regulatory review process including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure and a reduction or elimination of registration and marketing authorization fees. During the period of market exclusivity, a marketing authorization may only be granted for a "similar medicinal product" with the same orphan indication as an authorized orphan medicinal product if: (i) the marketing authorization holder for the original orphan medicinal product consents to the authorization of the second medicinal product; (ii) the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities of the product; or (iii) it is established that the second product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The period of market exclusivity may, in addition, be reduced to six years if at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation because, for example, the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity.

Regulatory Requirements After a Marketing Authorization has been Obtained

Where an authorization for a medicinal product in the EU is obtained, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include:

- Compliance with the EU's stringent pharmacovigilance or safety reporting rules must be ensured. These rules can impose post-authorization studies and additional monitoring obligations.
- The manufacturing of authorized medicinal products, for which a separate manufacturer's license is mandatory, must also be conducted in strict compliance with the applicable EU laws, regulations and guidance, including Directive 2001/83/EC, Directive 2017/1572, Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice. These requirements include compliance with EU cGMP standards when manufacturing medicinal products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the EU with the intention to import the active pharmaceutical ingredients into the EU.
- The marketing and promotion of authorized medicinal products, including industry-sponsored continuing medical education and advertising directed toward the prescribers of medicinal products and/or the general public, are strictly regulated in the EU notably under Directive 2001/83/EC83EC, as amended, and are also subject to EU Member State national laws. Direct-to-consumer advertising of prescription medicines is prohibited across the EU.

The aforementioned EU rules are generally applicable in the EEA.

Reform of the Regulatory Framework in the European Union

The European Commission introduced legislative proposals in April 2023 that, if implemented, will replace the current regulatory framework in the EU for all medicines (including those for rare diseases and for children). The European Commission has provided the legislative proposals to the European Parliament and the European Council for their review and

approval and in April 2024, the European Parliament proposed amendments to the legislative proposals. Once the European Commission's legislative proposals are approved (with or without amendment), they will be adopted into EU law.

Data Protection Regulation in the European Economic Area and United Kingdom

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EEA, including personal health data, is subject to the EU General Data Protection Regulation, or EU GDPR and similar processing of personal data regarding individuals in the UK is subject to the UK General Data Protection Regulation, or UK GDPR, and the UK Data Protection Act 2018. In this Annual Report, GDPR refers to both the EU GDPR and the UK GDPR, unless specified otherwise. 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 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 also imposes strict rules on the transfer of personal data to countries outside the EEA/UK, including the U.S., and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million (£17.5 million) or 4% of annual global revenues, whichever is greater. The GDPR 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. Compliance with the GDPR will be a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance.

Brexit and the Regulatory Framework in the United Kingdom

The UK formally left the EU on January 31, 2020. As a result of the Northern Ireland Protocol, following Brexit, the EMA remained responsible for approving novel medicines for supply in Northern Ireland under the EU centralized procedure, and a separate authorization was required to supply the same medicine in Great Britain (England, Wales and Scotland). A new framework named the Windsor Framework was approved by the EU-UK Joint Committee on March 24, 2023, and the medicines aspects of the Windsor Framework have applied since January 1, 2025. This new framework fundamentally changes the previous system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the UK. The MHRA is now responsible for approving all medicinal products destined for the UK market (i.e., Great Britain and Northern Ireland) and the EMA no longer has any role in approving medicinal products destined for Northern Ireland under the EU centralized procedure. A single UK-wide marketing authorization will be granted by the MHRA for all novel medicinal products to be sold in the UK, enabling products to be sold in a single pack and under a single authorization throughout the UK. However, although a separate authorization is now required to market medicinal products in the UK, under an international recognition procedure which was put in place by the MHRA on January 1, 2024, the MHRA may take into account decisions on the approval of a marketing authorization from the EMA (and certain other regulators) when considering an application for a UK marketing authorization. There is now no pre-marketing authorization orphan designation in the UK. Instead, the MHRA reviews applications for orphan designation in parallel to the corresponding MAA. The criteria are essentially the same, but have been tailored for the UK market, i.e., the prevalence of the condition in the UK (rather than the EU) must not be more than five in 10,000. Should an orphan designation be granted, the period of market exclusivity will be set from the date of first approval of the product in the UK.

Pricing Decisions for Approved Products

In the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional cost effectiveness assessments that compare the cost-effectiveness of a particular product candidate to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, EU Member States have the option to restrict the range of 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 products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage health care expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on health care costs in general, particularly prescription products, has become intense.

As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU Member States, and parallel trade, i.e., arbitrage between low-priced and high-priced EU Member States, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any products, if approved in those countries.

Rest of the World Regulation

For other countries outside of the EU and the U.S., such as countries in the Middle East, Africa, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. Additionally, the clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Coverage and Reimbursement

Successful commercialization of new drug products depends in part on the extent to which reimbursement for those drug products will be available from government health administration authorities, private health insurers, and other organizations. In the U.S., government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drug products they will pay for and establish reimbursement levels. The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford a drug product. Sales of drug products depend substantially, both domestically and abroad, on the extent to which the costs of drugs products are paid for by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular drug products. Third-party payors are increasingly challenging the price, examining the medical necessity, and reviewing the cost-effectiveness of medical products, therapies and services, in addition to questioning their safety and efficacy. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with branded drugs and drugs administered under the supervision of a physician. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA and foreign approvals. These studies could result in delays or disadvantageous coverage for products we develop. Our product candidates may not be considered medically necessary or cost-effective. 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 product on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. A payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on its investment in product development. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate that we successfully develop.

In many countries, the prices of drug products are subject to varying price control mechanisms as part of national health systems. In general, the prices of drug products under such systems are substantially lower than in the U.S. Other countries allow companies to fix their own prices for drug products, but monitor and control company profits. Accordingly, in markets outside the U.S., the reimbursement for drug products may be reduced compared with the U.S.

In the U.S., the principal decisions about reimbursement for new drug products are typically made by CMS, an agency within the HHS. CMS decides whether and to what extent a new drug product will be covered and reimbursed under Medicare, and private payors tend to follow CMS to a substantial degree. However, no uniform policy of coverage and reimbursement for drug products exists among third-party payors and coverage and reimbursement levels for drug products

can differ significantly from payor to payor. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a drug product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

We cannot be sure that coverage or reimbursement will be available for any product that we commercialize and, if coverage and reimbursement are available, what the level of reimbursement will be. Coverage may also be more limited than the purposes for which the product is approved by the FDA or comparable foreign regulatory authorities. Future coverage and reimbursement may be subject to increased restrictions, such as prior authorization requirements, and to changes in the rates of reimbursement for orphan drug products both in the U.S. and in international markets. Reimbursement may impact the demand for, or the price of, any product for which we obtain regulatory approval.

The MMA established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. While all Medicare drug plans must give at least a standard level of coverage set by Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each Part D prescription drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for drugs for which we obtain marketing approval. Any negotiated prices for any of our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the average manufacturer price, or AMP, and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although under the current state of the law these newly eligible entities (with the exception of children's hospitals) will not be eligible to receive discounted 340B pricing on orphan drugs. As the required 340B discount is determined based on average manufacturer price, or AMP, and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase. The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. The plan for the research was published in 2012 by HHS, the Agency for Healthcare Research and Quality and the NIH, and periodic reports on the status of the research and related expenditures are made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our product candidates, if any such drug or the condition that they are intended to treat are the subject of a trial. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's drug could adversely affect the sales of our product candidate. If third-party payors do not consider our drugs to be cost-effective compared to other available therapies, they may not cover our drugs after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our drugs on a profitable basis.

These laws and future state and federal healthcare reform measures may be adopted in the future, any of which may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Outside of the U.S., the pricing of pharmaceutical products is subject to governmental control in many countries. For example, in the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that

products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional cost effectiveness assessments that compare the cost effectiveness of a particular therapy to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. Other countries may allow companies to fix their own prices for products, but monitor and control product volumes and issue guidance to physicians to limit prescriptions. Efforts to control prices and utilization of pharmaceutical products will likely continue as countries attempt to manage healthcare expenditures.

Employees and Human Capital

As of December 31, 2024, we had 123 full-time employees. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider the relationship with our employees to be good.

Our human capital is integral to helping us achieve our goal to end the suffering caused by neurodegenerative diseases and endocrine conditions. The objectives for our human capital resources include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees and directors through the granting of stock-based compensation awards and cash-based performance bonus awards.

Available Information

We file Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, proxy statements and other information with the SEC. Our filings with the SEC are available on the SEC's website at www.sec.gov. We also maintain a website at http://www.amylyx.com. We make available, free of charge, in the Investors section of our website, documents we file with or furnish to the SEC, including our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and any exhibits and amendments to those reports. We make this information available as soon as reasonably practicable after we electronically file such materials with, or furnish such information to, the SEC. The other information found on our website is not part of this or any other report we file with, or furnish to, the SEC.

Environmental, Social, and Governance (ESG)

The values that drive our mission to develop novel therapies for communities with high unmet needs are at the heart of how we do business. Our commitment to audacity, curiosity, engagement, accountability, and authenticity compels us to be responsible members of the global community. In 2024, we adopted a formal ESG charter to support our ongoing commitment to environmental, health and safety, corporate social responsibility, corporate governance, sustainability, and other related trends, issues, and concerns relevant to the Company. The ESG Committee, formed in 2023, helps the Executive Leadership Team to develop strategy relating to ESG matters and support the integration of strategically significant ESG policies into the Company's business operations and strategy.

Given our reliance on outsourced operations, it is important that we select partners whose standards align with our own. Through our procurement department, we request information on our suppliers' practices and their commitment to sustainability. In 2024, we adopted a Supplier Code of Conduct. This code, which is posted on our corporate website, is an extension of Amylyx's Code of Business Conduct & Ethics and summarizes the legal obligations and ethical standards that we expect our suppliers to comply with.

Environmental

Most of our employees choose to work remotely, but we maintain features such as recycling programs and automatic lighting at our facilities to minimize our impact. We also expect our suppliers to operate in a responsible and efficient manner to minimize adverse impacts on the environment, including by conserving natural resources, engaging in reuse and recycling programs, and avoiding the use of hazardous materials where possible. We retain the ability to conduct periodic audits to review these programs.

Social

Every day, the communities we collaborate with drive us forward. We strive to be partners of choice to the individuals that we're humbled to work alongside and in service of. We've seen how heightened awareness and collaboration in partnership with the community and advocacy partners ignites innovation. With the growing availability of new

treatments, research, and technologies, we are dedicated to leading this movement in neurodegenerative and endocrine diseases.

As collaborators rooted in connection, we ask for input from the community early and often. We push through barriers in the treatment journey in an effort to generate breakthroughs that will make a real difference. For example, disease journeys may begin with the common problem of obtaining a correct diagnosis – for some, this takes years. Our approach to drug development accounts for the fast disease progression following long diagnosis timelines, significant heterogeneity in patient populations, the need to deepen the understanding of pathophysiology, and lack of consensus on measures of clinical benefit in drug development. Taking into consideration the complex ecosystem of addressing unmet medical needs allows us to focus on those areas where we can make the greatest impact.

We continually seek input on our clinical trial designs and desired outcomes, and recruitment and retention strategies by directly engaging with the patient communities we hope to serve. As we advance novel drug candidates in our pipeline, we will attempt to ensure equal access for all to clinical trials and, in particular, we will attempt to conduct clinical trials comprised of a diverse set of people impacted by the disease.

As an employer, diversity is also important, including having representation of diverse views and backgrounds at the highest levels of the organization. Three of our eight senior executives are women, and two of our seven board members are women.

We care deeply about supporting and investing in our people. Employee benefits are an important part of total rewards, and we're pleased to offer a comprehensive package to enable our employees' best work and help support their health, family, and way of life.

Our range of medical plans provides comprehensive coverage for our employees and their families with the flexibility to choose what will best suit their needs. We also offer dental and vision insurance. We complement these offerings with options for a Flexible Spending Account, or FSA, or Health Savings Account, or HSA for eligible medical expenses, Dependent Care FSA for child or elder care expenses, and a Limited Purpose FSA for dental and vision expenses.

In addition to the competitive benefits above, we offer:

•Hybrid Work Environment: We are primarily remote but have opportunities to come together as a full organization

•Touchpoints Rooted in Connection: Our in-person Anchor Weeks and bi-weekly Amylyx Exchange calls help us collaborate and connect with each other and the communities we're serving

•Learning and Development Program: Opportunities to grow professionally and network

•Recognition Platform: Recognizes employees beyond just the day-to-day

•"Take It As You Need It" Paid Time Off: Paid time off designed with flexibility for vacation, personal needs, school events, or appointments

•Employee Assistance Program: Access to mental health support, work-life solutions, financial resources, and legal guidance

•Paid Parental Leave: Ability to take time off and connect with your new child

•Mobile Reimbursement: Monthly cell phone reimbursement to stay connected

We affirm our commitment to a diverse and inclusive workplace by ensuring equitable compensation for all employees and upholding the principles of pay equity across all levels of the organization. We believe that fostering a diverse and inclusive environment is essential in providing a wide range of perspectives, and we are dedicated to continuously evaluating and evolving our practices to provide equitable access to learning, mentorship, leadership roles and career advancement opportunities.

Governance

Our Board of Directors is responsible for overseeing the business and management of the Company. As part of our governance practices, we are committed to high standards of ethics, which are reflected in our Code of Business Conduct and Ethics, which applies to our directors, officers, employees and designated agents. This Code is posted on our corporate

website. We have an independent chairman, and five of our seven board members are independent. Our Audit, Nominating and Corporate Governance, and Compensation Committees are comprised solely of independent directors.

Item 1A. Risk Factors.

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

Summary of Risk Factors

Risks Related to Our Financial Position and Need for Capital

- We have ceased marketing and selling our only commercial product in the U.S. and Canada and will therefore not continue to generate revenue from this product for the treatment of ALS. We expect to generate significant losses for the foreseeable future.
- We will not return to profitability unless and until we successfully commercialize any of our current or future product candidates.
- Our quarterly and annual operating results may fluctuate in the future. As a result, we may fail to meet or exceed the expectations of research analysts or investors, which could cause our stock price to decline and negatively impact our financing or funding ability, as well as negatively impact our ability to exist as a standalone company.
- We may require substantial additional funding in the future to meet our financial needs and to pursue our business objectives. If we are unable to obtain funding if and when needed, we could be forced to delay, reduce or eliminate our product discovery and development activities or commercialization efforts.

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

- We currently depend heavily on the success of avexitide, our most advanced product candidate, and AMX0035. If we are unable to successfully complete late-stage trials, obtain regulatory approvals for, and successfully commercialize, avexitide and/or AMX0035, or experience significant delays in doing so, our business may be materially harmed.
- The delay or denial of regulatory approval for any of our current or any future product candidates in any jurisdiction could adversely impact our business and our results of operations, and could cause us delay or even cease operations.
- We have historically concentrated our research and development efforts on the treatment of neurodegenerative diseases, a field that has seen very limited success in product development, and have only recently further expanded upon our existing development efforts in the endocrine and metabolic field, an area in which we have limited experience in drug development.
- The regulatory approval processes of the FDA and other comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to maintain or obtain regulatory approval for any current or future product candidates, our business will be substantially harmed.

Risks Related to Our Dependence on Third Parties

- We rely on third parties to assist in conducting our clinical trials. If they do not perform satisfactorily, we may not be able to obtain regulatory approval or successfully commercialize avexitide, AMX0035 or any other current or future product candidates, or such approval or commercialization may be delayed, and our business could be substantially harmed.
- We may seek to establish additional collaborations and if we are not able to establish and maintain them on commercially reasonable terms, we may have to alter our development and future commercialization plans.
- We have entered and may in the future enter into collaborations with third parties for the development and commercialization of avexitide, AMX0035 or any other current or future product candidates, and our prospects with respect to avexitide, AMX0035 and our other current or future product candidates will depend in significant part on the success of those collaborations.

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

- The markets for avexitide for PBH, and congenital HI, for AMX0035 for Wolfram syndrome, PSP and other neurodegenerative diseases, and for any other product candidates we are currently developing or may in the future develop or acquire may be smaller than we expect.
- If we are unable in the future to expand our sales, marketing, manufacturing and distribution capabilities or enter into agreements with third parties to market and sell avexitide, AMX0035 or other current or future product candidates for which we obtain marketing approval, we will be unable to generate any additional product revenue.
- Even if any current or future product candidate of ours receives regulatory approval, it may fail to maintain the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for continued commercial success or to remain profitable.

Risks Related to Our Intellectual Property

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

Risks Related to Our Business Operations and Employee Matters

- We are currently operating in a period of economic uncertainty and capital markets disruption, which has been significantly impacted by geopolitical instability, ongoing military conflicts, including the ongoing war between Russia and Ukraine, the Israel-Hamas conflict and conflict in the Middle East, a new U.S. presidential administration and accompanying regulatory activities and economic policies, events related thereto, and changes in inflation and interest rates, any of which could have a material adverse effect on our business, financial condition and results of operations.
- We depend heavily on our executive officers, principal consultants and others, and the loss of their services would materially harm our business.
- We only have a limited number of employees to manage and operate our business.
- We are continuously evaluating and pursuing strategic transactions, and may pursue strategic transactions in the future that are aligned with our mission to improve our underlying business performance.

Risks Related to Our Common Stock

- Unfavorable macroeconomic conditions or market volatility resulting from national or global economic conditions, including those affecting the financial services industry, could adversely affect our business, financial condition or results of operations.

Risks Related to Our Financial Position and Need for Capital

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

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We invested substantial resources into our product development efforts of AMX0035, and toward the commercialization of AMX0035 as RELYVRIO for ALS, which was approved by the FDA, and ALBRIOZA and, received marketing authorization with conditions from Health Canada. We voluntarily discontinued the marketing authorizations for RELYVRIO/ALBRIOZA for ALS and removed the product from the market in the U.S. and Canada based on topline results from the Phase 3 PHOENIX trial, which failed to meet its prespecified primary and secondary endpoints. Following the withdrawal of RELYVRIO/ALBRIOZA from the market in the US and Canada, respectively, we do not have any products approved for commercial sale and we will continue to incur significant research and development and other expenses related to clinical development and potential approvals for our current and future product candidates, including for avexitide, AMX0035 in additional indications other than ALS, and for ongoing operations. Since our inception, we have devoted the majority of our financial resources and efforts to research and development, including preclinical studies and our clinical trials, preparation for commercialization and commercialization activities. Our financial condition and operating results, including our revenues, expenses and net income (loss), have in the past and are likely in the future to fluctuate significantly from quarter to quarter and year to year. For example, we generated revenues of $380.8 million in 2023 as a result of sales of RELYVRIO/ALBRIOZA, but following its withdrawal, we will no longer generate revenues from this product. Accordingly, you should not rely upon the results of any prior quarterly or annual periods as indications of future operating performance. Additionally, net losses and negative cash flows have had, and may in the future have, an adverse effect on our stockholders' equity and working capital. As of December 31, 2024, we had an accumulated deficit of $606.7 million.

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

- conduct clinical trials of avexitide, AMX0035 and any other current or future product candidates;
- seek to identify and advance additional product candidates;
- initiate and continue research, preclinical and clinical development efforts for any current or future product candidates;
- add operational, financial and management information systems and personnel, including personnel to support commercialization of any current or future product candidate development and to help us comply with our obligations as a public company;
- maintain, expand and protect our intellectual property portfolio;
- seek regulatory approvals for avexitide or AMX0035 in indications that successfully complete clinical development; and
- acquire or in-license other product candidates, products or technologies.

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

Our ability to once again become and remain profitable depends on our ability to generate revenue. While we have a limited history of generating revenue from the commercialization of RELYVRIO/ALBRIOZA, we do not expect to generate additional significant revenue, if any, unless and until we, either alone or with a collaborator, are able to obtain regulatory approval for, and successfully commercialize, avexitide, AMX0035 for indications other than ALS or any other current or future product candidates or products we may develop or in-license. Successful commercialization will require achievement of many key milestones, which vary by jurisdiction and may include demonstrating safety and efficacy in clinical trials, obtaining regulatory, including marketing, approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our future collaborators, may obtain regulatory approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately and precisely predict the timing and amount of revenues, if any, 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 resume revenue generating activities or regain profitability may continue to depress the market price of our common stock and could impair our ability to raise capital or obtain other financing, expand our business, diversify our product offerings or continue our operations. If we continue to suffer losses as we have in the past, prior to the commercialization of RELYVRIO and ALBRIOZA, investors may not receive any return on their investment and may lose their entire investment.

Our quarterly and annual operating results may fluctuate in the future. As a result, we may fail to meet or exceed the expectations of research analysts or investors, which could cause our stock price to decline and negatively impact our financing or funding ability, as well as negatively impact our ability to exist as a standalone company.

Our financial condition and operating results have varied in the past and will continue to fluctuate from quarter-to-quarter and year-to-year in the future due to a variety of factors, many of which are beyond our control. Factors relating to our business that may contribute to these fluctuations include the following, as well as other factors described elsewhere in this Annual Report:

- our ability to manufacture and deliver supply of avexitide or AMX0035;
- our ability to maintain market acceptance of our product and product candidates, if approved, as a treatment option;
- delays or failures in advancement of existing or future development candidates into the clinic or product candidates in clinical trials;
- the feasibility of developing, manufacturing, and commercializing our product and product candidates;
- our ability to manage our growth;
- the outcomes of research programs, clinical trials or other product development or approval processes;
- our ability to successfully develop avexitide and AMX0035 for additional indications and to commercialize avexitide and AMX0035 for such additional indications, if approved;
- risks associated with the international aspects of our business including the conduct of clinical trials in multiple locations and potential commercialization in such locations;
- our ability to accurately report our financial results in a timely manner;
- our dependence on, and the need to attract and retain, key management and other personnel;
- our ability to obtain, protect and enforce our IP rights;
- our ability to prevent the theft or misappropriation of our IP, know-how or technologies;
- advantages that our competitors and potential competitors may have in securing funding, obtaining the rights to critical IP or developing competing technologies or products;
- our ability to obtain additional capital that may be necessary to expand our business;
- business interruptions such as power outages, strikes, acts of terrorism or natural disasters; and
- the ultimate impact of global economic and geopolitical events.

Due to the various factors mentioned herein, and others, the results of any of our prior quarterly or annual periods should not be relied upon as indications of our future operating performance.

Our financial results may fluctuate significantly from quarter-to-quarter and year-to-year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline. Our stock price may also decline as a result of unexpected clinical trial results in one or more of our ongoing or future clinical trials.

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

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

Our future capital requirements depend on many factors, including:

- the scope, progress, results and costs of researching and developing avexitide, AMX0035 for Wolfram syndrome, PSP and potential additional indications, AMX0114, as well as any other product candidates we are currently developing or may in the future develop;
- the timing of, and the costs involved in, our efforts to obtain marketing approvals for avexitide in PBH, AMX0035 for the treatment of Wolfram syndrome, PSP and potential additional indications, AMX0114, and our efforts to obtain approvals for other product candidates we are developing or may in the future develop and pursue;
- the number of other product candidates that we may pursue and their development requirements;
- the costs of commercialization activities for avexitide, AMX0035 and 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 avexitide and AMX0035 for any approved indications or any other current or future product candidates;
- the extent to which we in-license, acquire, or acquire rights to other products, product candidates or technologies;
- our obligation, if any, to pay royalties in connection with the development and commercialization of avexitide or any other products or product candidates we may in-license or acquire;
- our headcount fluctuation;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights, including enforcing and defending intellectual property related claims; and
- the ongoing costs of operating as a public company.

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

We have no committed source of additional capital and if we are unable to raise additional capital or secure other financing, if needed, in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development of avexitide, AMX0035 or any other current or future product candidates or other research and development initiatives. We may need to seek collaborators for avexitide, AMX0035 and any other current or future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be

available or relinquish or license on unfavorable terms our rights to avexitide, AMX0035 and any other current or future product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves. Any of the above events could significantly harm our business, prospects, financial condition, and results of operations and cause the price of our common stock to decline.

We believe our existing cash, cash equivalents and marketable securities as of December 31, 2024, along with the proceeds from the January follow-on 2025 offering ("January 2025 Offering"), will be sufficient to meet our anticipated operating and capital expenditure requirements through 2026. However, our estimate may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned.

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

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

To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, your ownership interest may be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. In addition, debt financing, if available, may result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. Securing financing could also require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of avexitide, 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, local and international income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U.S. Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

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

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

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

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

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

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

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

Our future success depends significantly on our ability to successfully develop, and obtain regulatory approvals for and commercialize, avexitide in PBH and AMX0035 in indications other than ALS, including Wolfram syndrome and PSP. Avexitide has been evaluated in five Phase 2 clinical studies for PBH and Congenital HI. In February 2025, we activated the first sites for the pivotal Phase 3 LUCIDITY clinical trial for avexitide in PBH. To date, we have obtained limited clinical trial data supporting AMX0035 in indications other than ALS, having only completed a clinical trial in 95 patients with AD. We are conducting a Phase 2 clinical trial of AMX0035 in Wolfram syndrome, and a global Phase 3 clinical trial of AMX0035 in PSP, and intend to conduct additional clinical trials for other indications and product candidates in the future. Our business success depends heavily on our ability to successfully complete clinical trials for our product candidates. We have completed the IND enabling studies of AMX0114 in ALS and the first site was activated for a Phase 1 multiple ascending dose, placebo-controlled trial in Canada in February 2025.

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

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

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

- the interpretation of our preclinical and clinical data by regulatory authorities to support marketing approvals;
- potential unforeseen safety issues or adverse side effects;
- receipt and maintenance of marketing approvals from applicable regulatory authorities, including with any expected NCE and new clinical investigation data exclusivity and orphan drug market exclusivity, as applicable;
- receipt and maintenance of designations from applicable regulatory authorities, including breakthrough designation for avexitide and orphan designation for avexitide and AMX0035;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for avexitide, AMX0035 or any other current or future product candidates;
- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of avexitide, AMX0035 or any other current or future product candidates;
- entry into collaborations to further the development of avexitide, AMX0035 or any other current or future product candidates;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- acceptance of avexitide, AMX0035 or any other products, if and when approved, by patients, the medical community and third-party payors;
- appropriately identifying patients with the diseases targeted by avexitide, AMX0035 or any other current or future product candidates and accurately estimating the size of applicable patient populations or disease prevalence;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- maintaining a continued acceptable safety profile of products following any approval;
- effectively competing with other drugs or therapies;
- ensuring that we promote and distribute our products consistent with all applicable healthcare laws; and
- enforcing and defending intellectual property rights and claims.

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

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

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

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

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

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

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

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

Under the FDA's combination rule, the FDA generally will not file or approve an NDA for a fixed-dose combination product unless each component of a proposed drug product is shown to make a contribution to the claimed effects and the dosage of each component (amount, frequency, duration) is safe and effective for the intended population. For additional information on FDA's combination rule, see the section entitled "*Business—Government Regulation—Combination Rule for Fixed-Dose Combination Products*" in our 2024 Annual Report.

Similar requirements may be imposed on us by the EMA in the EU and comparable regulatory authorities in other jurisdictions where we intend to seek regulatory approval. For any fixed-dose combination products we may develop, we may be required to produce clinical data supporting the contribution of each component when present at the levels included in the fixed-dose combination in order to obtain marketing authorization in the U.S. or EU.

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

neurology, specifically within ALS, on our products or other products may influence regulatory decision making, including for fixed-dose combinations.

We have historically concentrated our research and development efforts on the treatment of neurodegenerative diseases, a field that has seen very limited success in product development, and have only recently further expanded upon our existing development efforts in the endocrine and metabolic field, an area in which we have limited experience in drug development.

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

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

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

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

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

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

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

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

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

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

Additionally, we may experience interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with any future outbreak of any highly infectious or contagious diseases. As a result of a future public health crisis, we may face delays in meeting our anticipated timelines for our ongoing and planned clinical trials.

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

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

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

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

- regulators, or institutional review boards, or IRBs, or other reviewing bodies may not authorize us or our investigators to commence a clinical trial, or to conduct or continue a clinical trial at a prospective or specific trial site;
- we may not reach agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the number of subjects or patients required for clinical trials of avexitide, 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 avexitide, AMX0035 or any other current or future product candidates or conducting clinical trials on our behalf, may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may have to amend clinical trial protocol submitted to regulatory authorities or conduct additional studies to reflect changes in regulatory requirements or guidance, which we may be required to resubmit to an IRB and regulatory authorities for re-examination;
- unforeseen safety events may occur during the course of a clinical trial and these events may result in the temporary suspension or termination of a clinical trial, or require urgent safety measures or restrictions to protect human subjects during the conduct of a clinical trial;
- regulators, IRBs or other reviewing bodies may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we enter into agreement for clinical and commercial supplies, or the supply or quality of avexitide, AMX0035 or any other current or future product candidate or other materials necessary to conduct clinical trials of avexitide, AMX0035 or any other current or

future product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and

•the potential for approval policies or regulations of the FDA or any other applicable foreign regulatory agencies to significantly change in a manner rendering our clinical data insufficient for approval.

Regulators, IRBs of the institutions in which clinical trials are being conducted or data monitoring committees may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. For example, we submitted an Investigational New Drug application to the FDA for AMX0114. The FDA restricted dosing to an amount that is lower than our proposed starting dose of 12.5 mg and has requested additional information, which resulted in a clinical hold. Toxicology studies showed a greater than 10X safety margin at the starting dose of 12.5 mg based on the no observed adverse effect level, or NOAEL determined by independent toxicology firms. In January 2025, we announced that the clinical hold had been lifted, however there can be no assurance that future clinical holds relating to any of our current or future product candidates will not be imposed.

Negative or inconclusive impressions of the results from earlier clinical trials of avexitide performed by Eiger or any other clinical trial or preclinical studies in animals that we or Eiger, with respect to avexitide, have conducted, or the results from our earlier clinical trials of AMX0035 for the treatment of ALS or AD, 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 avexitide, AMX0035 for our intended indications or any future product candidate.

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

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

develop may not be effective, may be only moderately effective, or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

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

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

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

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

Additionally, we have in the past utilized and may in the future utilize an "open-label" clinical trial design. An "open-label" clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge. The results from an open-label trial may not be predictive of future clinical trial results with avexitide, 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 avexitide, AMX0035 or any other current or future product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials to such trial's conclusion as required by the FDA or other comparable foreign regulatory authorities. Additionally, certain clinical trials for avexitide, AMX0035 and any other current or future product candidates may be focused on indications with relatively small patient populations, which may further limit enrollment of eligible patients or may result in slower enrollment than we anticipate. The eligibility criteria of our clinical trials, once established, may further limit the pool of available trial participants. For example, the number of patients suffering from Wolfram syndrome and PBH, is small and, in some cases, has not been established with precision. If the actual number of patients with these diseases is smaller than we anticipate, we may encounter difficulties in enrolling patients in our clinical trials, thereby delaying or preventing development and approval of avexitide, AMX0035 or any other current or future product candidates. Even once enrolled, we may be unable to retain a sufficient number of patients to complete any of our trials. Neurodegenerative diseases have particular challenges, including significant mobility issues, morbidities and other complications that have historically made retention in clinical trials more challenging. In the past, we have had discontinuations in our clinical trials, including in our CENTAUR trial and our PHOENIX trial, and their open label extensions. Discontinuations may occur in current or future trials and could result in delays of completion of our clinical trials and affect our ability to enroll additional patients in our clinical trials and impact the integrity of data from our clinical trials.

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

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

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

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

of clinical trials, require the repetition of one or more clinical trials, increase clinical trial costs, delay approval of avexitide, AMX0035 or any other current or future product candidates and jeopardize our ability to commence sales and generate revenue.

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

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

Avexitide, 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 avexitide, 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, denial or withdrawal of regulatory approval by the FDA or comparable foreign regulatory authorities. Results of our preclinical studies or clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. In clinical trials to date of avexitide, avexitide was generally well-tolerated. The most common AEs were injection site bruising, headache, and nausea; these occurred more often with placebo than either avexitide dose. However, there can be no guarantee that we would observe a similar tolerability profile of avexitide in future clinical trials or for other indications.

In clinical trials of AMX0035 to date, AMX0035 has been generally well-tolerated, with most common treatment-emergent AEs including diarrhea, abdominal pain, nausea, upper respiratory infection, constipation, headache, fatigue, proteinuria, and decreased appetite. In addition, it has been reported that patients experience a bad taste when taking AMX0035. In animal studies, administration of AMX0035 to rats throughout pregnancy and lactation resulted in increased offspring mortality at all doses tested, which were less than or similar to the clinical doses tested in our clinical trials. However, there can be no guarantee that we would observe a similar tolerability profile of AMX0035 in future clinical trials or for other indications.

Many compounds that initially showed promise in clinical or earlier stage testing are later found to cause undesirable or unexpected side effects that prevented further development of the compound. If unacceptable or severe side effects arise in the development of avexitide, AMX0035 or any other current or future product candidates, we, the FDA or comparable foreign regulatory authorities, the IRBs, or independent ethics committees at the institutions in which our trials are conducted, or the independent safety monitoring committee could suspend or terminate our clinical trials or regulatory authorities could order us to cease clinical trials or deny approval of avexitide, AMX0035 or any other current or future product candidates for any or all targeted indications. Treatment-emergent side effects that are deemed to be drug-related could also affect subject recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. Undesirable side effects in one of our clinical trials for AMX0035 in one indication or avexitide could adversely affect enrollment in clinical trials, regulatory approval and commercialization of AMX0035 in other indications or avexitide. Additionally, there may be negative findings regarding components of avexitide, AMX0035 or future product candidates by other parties. Any negative findings by third parties may impact the future approvability or labeling of avexitide, AMX0035 or other product candidates we may develop. In addition, side effects may not be appropriately recognized or managed by the treating medical staff. Inadequate training in recognizing or managing the potential side effects of avexitide, 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, or limit its commercial adoption. AMX0035 is a combination of TURSO and PB.

PB has been approved by the FDA and other regulatory authorities for the treatment of patients with certain urea cycle disorders and TURSO has been approved in Italy for diseases of cirrhotic liver disorders such as primary biliary cirrhosis. It is possible that one or more of the active moieties in AMX0035 has also been approved by FDA or other regulatory authorities. Even if AMX0035 receives marketing approval and is commercialized in a jurisdiction, we would continue to be subject to the risks that the applicable regulatory authorities could revoke approval of PB or TURSO or any active moiety in AMX0035, if applicable, or that efficacy, manufacturing or supply issues could arise with PB or TURSO or any active moiety in AMX0035, if applicable. This could result in our own products being removed from the market or being less commercially successful.

Finally, clinical trials of avexitide and 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 avexitide, AMX0035 or any other current or future product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects.

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

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

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

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

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

We are currently, and plan to continue to, develop and evaluate avexitide and evaluate AMX0035 in other indications other than ALS, to continue to develop other product candidates. We intend to evaluate internal opportunities from avexitide, 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. For example, we recently completed the acquisition of substantially all of the rights, title and interests in, to and under those assets and interests used by Eiger in the development, manufacture and commercialization of avexitide. Avexitide has been evaluated in five Phase 2 clinical studies for PBH and Congenital HI, both diseases with unmet need, and we intend to initiate a Phase 3 program in PBH.

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

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

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

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

We may not be successful in our efforts to expand our pipeline by identifying additional product candidates or indications and modifications for which to investigate avexitide or AMX0035 in the future. We may expend our limited resources to pursue particular product candidates or indications or formulations for avexitide or AMX0035 and fail to capitalize on such product candidates or indications or formulations of avexitide or AMX0035 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 avexitide, AMX0035 and AMX0114. As a result, we may fail to generate additional clinical development opportunities for such candidates for a number of reasons, including, that such candidates may in certain indications, on further study, be shown to have harmful side effects, limited to no efficacy, or other characteristics that suggest it is unlikely to receive marketing approval and achieve market acceptance in such additional indications.

We plan to conduct several clinical trials for AMX0035 in parallel over the next several years, including clinical trials in patients with Wolfram syndrome, PSP and other indications, which may make our decision as to which indication to prioritize more difficult. Moreover, we intend to conduct a clinical trial of avexitide in PBH and may conduct others in other indications as well. As a result, we may forgo or delay pursuit of opportunities with other indications that we believe could have had greater commercial potential or likelihood of success. In addition, we are continuing to evaluate plans to explore the use of AMX0035 in patients with AD, and other product candidates in ALS and additional neurodegenerative diseases. However, we may focus on or pursue one or more of our target indications over other potential indications and product candidates and such development efforts may not be successful, which would cause us to delay the clinical development and approval of avexitide, AMX0035 and other product candidates. Furthermore, research activities to identify additional indications for avexitide, AMX0035 and other product candidates require substantial technical, financial, and human resources. We may not successfully develop these additional modifications for chemistry-related, stability-related, or other reasons. We have completed the IND-enabling studies of AMX0114, a potent antisense oligonucleotide targeting inhibition of Calpain-2, a key contributor to the axonal (also known as Wallerian) degeneration pathway, and began the Phase 1 multiple ascending dose, placebo-controlled trial in Canada in February 2025. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development activities for specific indications or formulations of avexitide, AMX0035 or for AMX0114 or other product candidates may not yield any commercially viable products.

Additionally, we may pursue in-licenses or acquisitions of development-stage assets or programs, which entails additional risk to us. For example, we recently completed the acquisition of substantially all of the rights, title and interests in, to and under those assets and interests used by Eiger in the development, manufacture and commercialization of avexitide. Avexitide has been evaluated in five Phase 2 clinical studies for PBH and congenital HI, both diseases with unmet need, and we initiated a Phase 3 program in PBH in February 2025. Identifying, selecting and acquiring promising product candidates requires substantial technical, financial, and human resources expertise and, once acquired, requires us to devote substantial resources. Efforts to do so may not result in the actual acquisition or license of a particular product candidate, and, if

acquired, may results in extensive diligence and preparation efforts, each of which may potentially result in a diversion of our management's time and the expenditure of our resources with no resulting benefit.

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

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

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

Many of our competitors have significantly greater financial resources, established presence in the market, expertise in research and development, manufacturing, preclinical and clinical testing, obtaining regulatory approvals and reimbursement and marketing approved products than we do. Accordingly, our competitors may be more successful than we may be in obtaining regulatory approval for therapies and achieving widespread market acceptance. Our competitors' products may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render avexitide, AMX0035 or any future product candidates obsolete or non-competitive before we can recover development and commercialization expenses. If avexitide or 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 products that are more effective or less costly than avexitide, AMX0035 or any future product candidates that we may develop, which could render such product candidates obsolete and noncompetitive.

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

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

Obtaining and maintaining regulatory approval of avexitide, 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 avexitide, 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. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S. and other jurisdictions, including additional preclinical studies or clinical trials, as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the U.S., including Canada, and certain jurisdictions in the EU, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

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

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

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

We received orphan drug status for AMX0035 for the treatment of patients with Wolfram syndrome in the U.S. in November 2020. Eiger received orphan drug status for avexitide for the treatment of hyperinsulinemic hypoglycemia (which includes PBH and Congenital HI) in the U.S. in December 2016 and for Congenital HI in Europe from the European Commission in November 2019. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the other regulatory bodies from approving another marketing authorization application for the same drug for that time period. Another drug may receive marketing approval prior to avexitide or AMX0035. The applicable period is seven years in the U.S. and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory agency's agreed upon pediatric investigation plan. The exclusivity period in the EU may be reduced to six years if, at the end of the fifth year, it is demonstrated that a product no longer meets the criteria for orphan designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. In the EU, during the ten-year period of orphan marketing exclusivity, neither the competent authorities of the EU Member States, the EMA, or the European Commission are permitted to accept applications or grant marketing authorization for similar medicinal products to the authorized orphan product. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. Legislation has been proposed by the European Commission that, if implemented, has the potential in some cases to shorten the ten-year period of

orphan marketing exclusivity. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA and the EMA can subsequently approve another drug for the same condition before the expiration of the seven-year (or ten-year in the EU) exclusivity period if the FDA or the EMA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In addition, if an orphan designated product receives marketing approval for an indication broader than or different from what is designated, such product may not be entitled to orphan exclusivity. Even though the FDA has granted orphan drug designation to AMX0035 for the treatment of Wolfram syndrome, if we receive approval for AMX0035 for a modified or different indication, our current orphan designation may not provide us with exclusivity.

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

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

We may pursue orphan drug designation for avexitide or 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 U.S., or a patient population of greater than 200,000 people in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. If our incidence or prevalence estimates for future indications for which we may seek orphan drug designation are incorrect, we may be unable to receive orphan drug designation.

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

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

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A Priority Review Designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. For example, we received priority review for AMX0035 for the treatment of ALS, and we may in the future request Priority Review Designation for any future product candidates, however, we cannot assume that any application for future indications of avexitide, 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 the treatment of ALS, for June 29, 2022, and then extended the review timeline for our NDA to September 2022. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

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

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

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

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

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe a product candidate we develop meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of Breakthrough Therapy Designation for a product candidate, such as avexitide, may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if any product candidate we develop qualifies as a breakthrough therapy, the FDA may later decide that the drug no longer meets the conditions for qualification and rescind the designation.

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

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

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

•the NDA is approved for a different adult indication than the rare pediatric disease for which avexitide is designated.

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

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

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

•decreased demand for any of our future approved products;

•injury to our reputation;

•withdrawal of clinical trial participants;

•termination of clinical trial sites or entire trial programs;

•significant litigation costs, including with respect to potential class action lawsuits;

•substantial monetary awards to, or costly settlements with, patients or other claimants;

•product recalls or a change in the indications for which they may be used;

•loss of revenue;

•diversion of management and scientific resources from our business operations; and

•the inability to commercialize avexitide, AMX0035 or any other current or future product candidates.

Although the clinical trial process is designed to identify and assess potential side effects, clinical development does not always fully characterize the safety and efficacy profile of a new drug, and it is always possible that a drug, even after regulatory approval, may exhibit unforeseen side effects. If avexitide, AMX0035 or any other current or future product candidates was to cause adverse side effects during clinical trials or after approval, we may be exposed to substantial liabilities. Physicians and patients may not comply with any warnings that identify known potential adverse effects and patients who should not use avexitide, 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 $10.0 million in the aggregate, including clinical trial liability, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We may need to increase our insurance coverage as we commercialize avexitide or AMX0035 in the U.S. and other jurisdictions, if approved, or any other current or future product candidate that receives regulatory approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and

sale of avexitide, AMX0035 or any other current or future product candidates, which could harm our business, financial condition, results of operations and prospects.

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

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

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

Accordingly, in any jurisdiction where we or any future collaborators, receive regulatory approval for avexitide, 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 avexitide, AMX0035 or any other current or future products withdrawn by regulatory authorities and our, or any future collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

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

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the U.S. to comply with certain accounting provisions requiring us to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

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

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

developing, manufacturing, or selling certain products and product candidates outside of the U.S., which could limit our growth potential and increase our development costs.

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

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

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

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

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

Risks Related to Our Dependence on Third Parties

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

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

Further, although our reliance on these third parties for clinical development activities limits our control over these activities, we remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. Moreover, the FDA and competent authorities of the EU Member States require us to comply with Good Clinical Practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and IRBs. If we or our third-party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or other regulatory body may require us to perform additional clinical trials before approving avexitide or AMX0035, including for additional indications, or any

other current or future product candidates, which would delay the regulatory approval process. We cannot be certain that, upon inspection, the FDA or other regulatory body will determine that any of our clinical trials comply with GCPs. For example, our clinical trial sites and investigators have in the past and may in the future engage in protocol deviations which could impact the overall interpretability of the outcomes of our clinical trials. We are also required to register certain clinical trials and post the results of completed clinical trials on a government-sponsored database, such as ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

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

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

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

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

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA or other comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available for collaboration and whether such a collaboration could be more attractive than the one with us for our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop avexitide, AMX0035, or any other current or future product candidates or bring them to market and generate product revenue.

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

one or more of our other development programs or activities, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or future commercialization activities at our own expense.

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

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

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of avexitide, 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 avexitide, AMX0035 or any of our other current or future product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, including trade secrets and intellectual property rights, contract interpretation, or the preferred course of development might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

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

Our use of third parties to manufacture avexitide or AMX0035 in compliance with cGMP may increase the risk that we will not have sufficient cGMP-compliant quantities of avexitide or AMX0035 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 avexitide or 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, or APIs, in AMX0035 and avexitide, and for the blending and packaging of AMX0035 and avexitide in accordance with applicable law, regulations and standards. Our current strategy is to outsource all manufacturing of AMX0035 and avexitide and any other current or future product candidates to third parties.

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

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

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

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

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

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

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

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

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

We may need to maintain licenses for active ingredients from third parties to develop and commercialize avexitide, 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 avexitide, AMX0035 or any other current or future product candidates that are proprietary to one or more third parties, we would need to maintain licenses to those active ingredients from those third parties. If we are unable to gain or continue to access rights to these active ingredients prior to conducting preclinical toxicology studies intended to support clinical trials, we may need to develop alternate product candidates from these programs by either accessing or developing alternate active ingredients, resulting in increased development costs and delays in commercialization of these product candidates. If we are unable to gain or maintain continued access rights to the desired active ingredients on commercially reasonable terms or develop suitable alternate active ingredients, we may not be able to commercialize product candidates from these programs.

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

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

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

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

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

To successfully commercialize any products that may result from our development activities or that we may acquire, we would need to continue to expand our sales, marketing, pharmacovigilance, manufacturing and distribution capabilities, either on our own or with others. RELYVRIO/ALBRIOZA, formerly sold in Canada and the U.S. for the treatment of ALS before being voluntarily withdrawn from the market, was the first product that we commercialized and built a global marketing and sales team for. The development of our own marketing and distribution effort was expensive and time-consuming and any efforts to do so in connection with our other product candidates may be expensive and time-consuming and could delay any further product launches. Moreover, we cannot be certain that we will be able to develop this capability successfully again in the future, despite our experience. We may enter into collaborations regarding any approved product candidates with other entities to utilize their established marketing and distribution capabilities, however, we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize avexitide, AMX0035 or any other current or future product candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We may also face competition in our search for

third parties to assist us with the sales and marketing efforts of avexitide, AMX0035 and any other current or future product candidates, if approved. Without an internal team or the support of a third-party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

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

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

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

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

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

Avexitide, AMX0035 or any future product candidates for which we, or any future collaborators, obtain regulatory approval in the future will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. If approved, avexitide, 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.

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

The FDA and other regulatory authorities may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. Additionally, the FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. Regulatory authorities impose stringent restrictions on manufacturers' communications regarding off-label use. However, companies generally may share truthful and not misleading information that is otherwise consistent with a product's approved labeling. If we, or any future collaborators, do not market avexitide, AMX0035 or any of our other current or future product candidates for which we, or they, receive regulatory approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing if it is alleged that we are doing so. Violation of laws and regulations relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws, including the False Claims Act and any comparable foreign laws. In the EU, the direct-to-consumer advertising of prescription-only medicinal products is prohibited. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public, if approved, and may also impose limitations on our promotional activities with health care professionals.

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

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

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

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

The success of avexitide, AMX0035 and any of our other current or future product candidates, if approved, depends on the availability of adequate coverage and reimbursement from third-party payors. Because avexitide, AMX0035 and any other current or future product candidates represent new approaches to the treatment of the diseases they target, we cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from, avexitide, AMX0035 and any other current or future product candidates or for any product that we may develop. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any such product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to any current or future product candidates we may develop (e.g., for the administration of our product candidate to patients) is also important. Inadequate reimbursement for such services may lead to physician and payor resistance and adversely affect our ability to market or sell avexitide, AMX0035 or any other current or future product candidates we may develop. In addition, we may need to develop new reimbursement models, in order to realize adequate value. Payors may not be able or willing to adopt such new models and patients may be unable to afford that portion of the cost that such models may require them to bear. If we determine such new models are necessary, but we are unsuccessful in developing them, or if payors do not adopt such models, our business, financial condition, results of operations and prospects could be adversely affected. For additional information on coverage and reimbursement, see the section entitled "*Business—Government Regulation—Coverage and Reimbursement*" in our 2024 Annual Report.

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

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

Outside the U.S., international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Canada, the EU and other countries has and will continue to put pressure on the pricing and usage of drug products such as avexitide, AMX0035 and any other current or future product candidates we may develop, if approved. We may also incur additional challenges when seeking reimbursement from public and private payers where avexitide, AMX0035 or any future product candidate has been approved subject to post-marketing conditions. Moreover, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product,

possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the U.S. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the U.S., the reimbursement for avexitide, AMX0035 and any other current or future product candidates we may develop may be reduced compared with the U.S. and may be insufficient to generate commercially reasonable revenues and profits.

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

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business. For more information, see the section entitled "*Business – Government Regulation – Current and Future U.S. Healthcare Reform Legislation*" in our 2024 Annual Report.

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

- the demand for avexitide, AMX0035 or any other current or future product candidates;
- our ability to set a price that we believe is fair for our approved products;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

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

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

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

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

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

In some countries, including Canada and certain Member States of the EU, the pricing of prescription drugs is, in part, subject to governmental control. Additional countries may adopt similar approaches to the pricing of prescription drugs. In

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

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

Healthcare providers, physicians and third-party payors in the U.S. and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, or FCA, which may constrain the business or financial arrangements and relationships through which such companies conduct research, sell, market and distribute pharmaceutical products. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. For more information, see the section entitled "*Business – Government Regulation - Other U.S. Healthcare Laws*" in our 2024 Annual Report.

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

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

complied with the law, a government investigation, including of any business partners, vendors or charitable foundations, could impact our business practices, harm our reputation, divert the attention of management, increase our expenses, and reduce the availability of foundation support for our patients who need assistance.

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

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

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

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

Further, legislation may be introduced that, if passed, would, among other things, further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting, and any additional future changes to the definition of average manufacturer price or the Medicaid

rebate amount could affect our 340B ceiling price calculations and negatively impact our results of operations. Additionally, certain pharmaceutical manufacturers are involved in ongoing litigation regarding contract pharmacy arrangements under the 340B program. For example, on November 3, 2023, the U.S. District Court of South Carolina issued an opinion in Genesis Healthcare Inc. v. Becerra et al. that may lead to an expansion of the scope of patients eligible to access prescriptions at 340B pricing. The outcome of this and other judicial proceedings on the 340B program and the potential impact on the way in which manufacturers extend discounts to covered entities through contract pharmacies under the 340B program remain uncertain.

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

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other information processing worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Economic Area, or, EEA, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, and similarly, processing of personal data regarding individuals in the United Kingdom, or the UK, including personal health data, is subject to the UK General Data Protection Regulation and the UK Data Protection Act 2018, or, collectively the UK GDPR, and together with the EU GDPR, the GDPR. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining the consent of the individuals to whom the personal data relates, providing detailed information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EEA/UK that are not considered by the European Commission and the UK government as providing "adequate" protection to personal data, including the U.S., and, as a result, increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the U.S. Such transfers of personal data outside of the EEA and UK are prohibited unless a valid GDPR transfer mechanism (for example, the European Commission approved Standard Contractual Clauses, or SCCs, and the UK International Data Transfer Agreement/Addendum, or UK IDTA) has been put in place. Where relying on the SCCs /UK IDTA for data transfers, we may also be required to carry out transfer impact assessments to assess whether the recipient is subject to local laws which allow public authority access to personal data. The international transfer obligations under the EEA/UK data protection regimes will require significant effort and cost, and may result in us needing to make strategic considerations around where EEA/UK personal data is transferred and which service providers we can utilize for the processing of EEA/UK personal data. Any inability to transfer personal data from the EEA and UK to the United States in compliance with data protection laws may impede our ability to conduct trials and may adversely affect our business and financial position. The GDPR also permits data protection authorities to require the destruction of improperly gathered or used personal information and or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or €20 million (£17.5 million under the UK GDPR), whichever is greater and it also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Although the UK is regarded as a third country under the EU GDPR, the European Commission has now issued a decision recognizing the UK as providing adequate protection under the EU GDPR, or Adequacy Decision, and, therefore, transfers of personal data originating in the EEA to the UK remain unrestricted. The UK Government has introduced a Data Protection and Digital Information Bill, or UK Bill, into the UK legislative process to reform the UK's data protection regime, and if passed, the final version of the UK Bill may have the effect of further altering the similarities between the UK and EEA data protection regimes and threaten the UK Adequacy Decision from the European Commission, which may lead to additional compliance costs for us and could increase our overall risk. It is unclear how UK data protection laws and regulations will develop in the medium to longer term, and how data transfers to and from the UK will be regulated in the long term. Although the EU GDPR and the EU GDPR currently impose substantially similar obligations, it is possible that over the time the UK GDPR could become less aligned with the EU GDPR. In addition, EU member states have adopted national laws to supplement the EU GDPR, which may partially deviate from the EU GDPR, and the competent authorities in the EU Member States may interpret EU GDPR obligations slightly differently from country to country, such that we do not expect to operate in a uniform legal landscape in the EEA with respect to data protection regulations. The potential of the respective provisions and enforcement of the EU GDPR and UK GDPR further diverging in the future creates additional regulatory challenges and uncertainties for us. The lack of clarity on future UK laws and regulations and their interaction with EU laws and regulations could add legal risk, uncertainty, complexity and cost to the

handling of European personal data and our privacy and data security compliance programs could require us to implement different compliance measures for the UK and EEA.

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

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

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

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

Risks Related to Our Intellectual Property

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

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

avexitide, as well as assuming Eiger's licenses to patents and applications directed to avexitide and owned and co-owned by other entities. The patent application process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

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

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

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

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

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

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the U.S., the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are not published until 18 months after filing, or in

some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patent or pending patent applications, or that we were the first to file for patent protection of such inventions. If third parties have filed prior patent applications on inventions claimed in our patents or applications that were filed on or before March 15, 2013, an interference proceeding in the U.S. can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such prior applications after March 15, 2013, a derivation proceeding in the U.S. can be initiated by such third parties to determine whether our invention was derived from theirs.

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

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

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

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

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance, whether intentional or not, can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, and sell avexitide, AMX0035 or AMX0114;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;

- countries other than the U.S. may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates; and
- countries other than the U.S. may, under certain circumstances, force us to grant a license under our patents to a competitor, thus allowing the competitor to compete with us in that jurisdiction or forcing us to lower the price of our drug in that jurisdiction.

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

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

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

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

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

We cannot provide assurances that any of our patent applications will be found to be patentable, including over our own prior art publications or patent literature, or will issue as patents. Neither can we make assurances as to the scope of any claims that may issue from our pending and future patent applications nor to the outcome of any proceedings by any potential third parties that could challenge the patentability, validity or enforceability of our patents and patent applications in the U.S. or

foreign jurisdictions. Any such challenge, if successful, could limit patent protection for our products and current or future product candidates and/or materially harm our business.

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

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

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

- we may not be able to generate sufficient data to support full patent applications that protect the entire breadth of developments in one or more of our programs;
- it is possible that one or more of our pending patent applications will not become an issued patent or, if issued, that the patent(s) claims will have sufficient scope to protect our technology, provide us with commercially viable patent protection or provide us with any competitive advantages;
- if our pending applications issue as patents, they may be challenged by third parties as invalid or unenforceable under U.S. or foreign laws;
- we may not successfully commercialize avexitide, AMX0035 or AMX0114 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 avexitide, AMX0035, or AMX0114 or any other current or future product candidates or in the event that such patent protection expires, it may no longer be cost-effective to extend our portfolio by pursuing additional development of a product or product candidate for follow-on indications.

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

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

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

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

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

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

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

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

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

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

In the EU, innovative medicinal products (including both small molecules and biological medicinal products), sometimes referred to as new active substances, or NAS, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents generic or biosimilar applicants from

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

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

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

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

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

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

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

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

Proceedings to enforce our patent rights, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Moreover, such proceedings could put our patents at risk of being invalidated or

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

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

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

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

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

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

proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources, and we may not have sufficient resources to bring these actions to a successful conclusion.

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

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

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

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

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

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

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

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

- others may be able to make products that are competitive to avexitide, AMX0035, for example a TURSO monotherapy, AMX0114 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, avexitide, AMX0035, AMX0114, and any other current or future product candidates. We also expect to collaborate with third parties on the development of avexitide,

AMX0035, AMX0114, and any other current or future product candidates. As a result of the aforementioned collaborations, we must, at times, share trade secrets with our collaborators.

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

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

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

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

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

Risks Related to Our Business Operations and Employee Matters

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

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

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

We are continuously evaluating and pursuing strategic transactions, and may pursue strategic transactions in the future that are aligned with our mission to improve our underlying business performance.

We anticipate completing acquisitions and business combinations in the future, especially given the discontinuation of the marketing authorizations for RELYVRIO/ALBRIOZA for ALS and its withdrawal from the commercial market in the U.S. and Canada. For example, in July 2024, we completed the acquisition of substantially all of the rights, title and interests in, to and under those assets and interests used by Eiger in the development, manufacture and commercialization of avexitide. Our ability to complete future acquisitions and business combinations will depend, in part, on the availability of suitable candidates at acceptable prices, terms, and conditions; our ability to compete effectively for acquisition candidates; and the availability of capital and personnel to complete such acquisitions and run the acquired business effectively. Any acquisition or business combination could impair our business, reputation, operating results and financial condition. The benefits of an acquisition or business combination may take more time than expected to develop or integrate into our operations, and we cannot guarantee that previous or future acquisitions or business combinations will, in fact, produce any benefits. For example, we may not receive the anticipated benefits of the acquisition of avexitide for some time. Acquisitions and business combinations may involve a number of risks, the occurrence of which could adversely affect our business, reputation, operating results and financial condition, including:

- diversion of management's attention;
- disruption to our existing operations and plans;
- inability to effectively manage our expanded operations;
- difficulties or delays in integrating and assimilating information and financial systems, operations, manufacturing processes and products of an acquired business or other business venture or in realizing projected efficiencies, growth prospects, cost savings, and synergies;
- inability to successfully integrate or develop a distribution channel for acquired product lines;
- potential loss of key employees, customers, distributors, or sales representatives of the acquired businesses or adverse effects on existing business relationships with suppliers, customers, distributors, and sales representatives;

•adverse impact on overall profitability if our expanded operations do not achieve the financial results projected in our valuation models;

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

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

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

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

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

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

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

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

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

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

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

Inadequate funding for the FDA, the SEC, the NIH 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.

Currently, federal agencies in the U.S. are operating under a continuing resolution that is set to expire on March 14, 2025 and the current U.S. administration is focused on reducing costs of the federal government generally, including significantly reducing the number of government employees. Without appropriation of additional funding to federal agencies, our business operations related to our product development activities for the U.S. market could be impacted. The ability of the FDA to review and approve new products and NIH's ability to conduct and partner with industry on important research 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, layoffs, 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, including executive and congressional priorities, 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. For example, over the last several years, the U.S. government has shut down and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. In addition, the current U.S. administration has proposed substantial reductions in force at various government agencies that, if applied in a material way, could significantly reduce the FDA's and other agencies' capacities to perform their functions in a manner consistent with past practices. If a prolonged government shutdown occurs or if the FDA, NIH or SEC experiences significant decreases in funding or personnel, it could significantly impact the ability of the FDA to issues licenses needed for conduct of our clinical trials, the NIH to conduct research or provide grants, and the abilities of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business and our timelines. 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.

The U.S. Congress, the Trump administration, or any new administration may make substantial changes to fiscal, tax, and other federal policies that may adversely affect our business.

Since the start of the Trump administration in 2025, U.S. policy changes have been implemented at a rapid pace and additional changes are likely. Changes to U.S. policy implemented by the U.S. Congress, the Trump administration or any new administration have impacted and may in the future impact, among other things, the U.S. and global economy, international trade relations, unemployment, immigration, healthcare, taxation, the U.S. regulatory environment, inflation and other areas. Although we cannot predict the impact, if any, of these changes to our business, they could adversely affect our business. Until we know what policy changes are made, whether those policy changes are challenged and subsequently upheld by the court system and how those changes impact our business and the business of our competitors over the long term, we will not know if, overall, we will benefit from them or be negatively affected by them.

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

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

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

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

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

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

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

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

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

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

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

Risks Related to Our Common Stock

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

The trading price of our common stock may be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. For example, from January 7, 2022, the first day that our stock traded on the Nasdaq Global Select Market, through December 31, 2024, our stock has traded within a range of a high price of $41.93 and a low price of $1.58 per share. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this Annual Report, these factors include:

- the commencement, enrollment or results of our ongoing and future preclinical studies and clinical trials, or any future preclinical studies or clinical trials, we may conduct of avexitide, AMX0035 and any other current or future product candidates, or changes in the development status of our current and any future product candidates;
- any additional regulatory submissions for avexitide, AMX0035 or any other current or future product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such submissions, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results or delays in our preclinical studies and clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial, or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approvals for avexitide, AMX0035 and any other current or future product candidates;
- withdrawal of products from the market;
- changes in laws or regulations applicable to current or future product candidates, including but not limited to clinical trial requirements for approvals;
- the failure to obtain coverage and adequate reimbursement of current or future product candidates, if approved;
- changes on the structure of healthcare payment systems;
- any changes to our relationship with any manufacturers, suppliers, licensors, future collaborators or other strategic partners;
- our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;
- our inability to establish collaborations, if needed;
- our failure to successfully commercialize avexitide, AMX0035 and any other current or future product candidates;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of avexitide, AMX0035 and any other current or future product candidates;
- introduction of new products or services offered by us or our competitors, or the release or publication of clinical trial results from competing product candidates;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us or our competitors;
- actual or anticipated variations in quarterly operating results;
- our cash position and rate of expenditures;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;

- overall performance of the equity markets;
- issuances of debt or equity securities;
- sales of our common stock by us or our stockholders in the future or the perception that such sales may occur;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political, geographical, and economic conditions, including the impact of global health crises such as the COVID-19 pandemic, historically high inflation, elevated interest rates, the ongoing wars in Ukraine and Israel and a new U.S. presidential administration and accompanying regulatory activities and economic policies; and
- other events or factors, many of which are beyond our control.

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

Unfavorable macroeconomic conditions or market volatility resulting from national or global economic conditions, including those affecting the financial services industry, could adversely affect our business, financial condition or results of operations.

Adverse macroeconomic conditions or market volatility resulting from national or global economic developments, political unrest, high inflation, elevated interest rates, changes in international trade relationships and military conflicts, such as the ongoing conflict between Russia and Ukraine, potential for significant changes in U.S. policies or regulatory environment or other factors, could materially and adversely affect our business operations. Sanctions imposed by the U.S. and other countries in response to such conflicts may also continue to adversely impact the financial markets and the global economy, and any economic countermeasures by the affected countries or others could exacerbate market and economic instability. There can be no assurance that deterioration in credit and financial markets and confidence in economic conditions will not occur. For instance, actual events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. Investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. In addition, any deterioration in the macro-economy or financial services industry could lead to losses or defaults by our suppliers, which in turn, could have a material adverse effect on our current and/or planned business operations and our current or projected results of operations and financial condition. Also, current inflationary trends in the global economy may impact salaries and wages, costs of goods and transportation expenses, among other things, and recent and potential future disruptions in access to bank deposits or lending commitments due to bank failures may create market and economic instability. A severe or prolonged economic downturn or additional global financial crises could result in a variety of risks to our business, including weakened demand for any product candidates we develop or our ability to raise additional capital when needed on acceptable terms, if at all.

Further, U.S. government appropriations have been affected by larger U.S. government budgetary issues and related legislation. Government spending levels are difficult to predict beyond the near term due to numerous factors, including the external threat environment, future government priorities and the state of government finances. Significant changes in government spending or changes in U.S. government priorities, policies and requirements could have a material adverse effect on our results of operations, financial condition or liquidity.

Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

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

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

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

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

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

As of June 30, 2023, the market value of our common stock that was held by non-affiliates exceeded $700 million, and therefore, effective as of January 1, 2024, we became a large accelerated filer. We also lost our status as an emerging growth company or smaller reporting company and were not able to avail ourselves of the reduced disclosure requirements available to smaller reporting companies. However, based on the market value of our common stock that was held by non-affiliates as of June 30, 2024, we became an accelerated filer, rather than a large accelerated filer, and we regained smaller reporting company status effective as of December 31, 2024 and have been able to avail ourselves of the reduced disclosure requirements. As a smaller reporting company, we are permitted and will rely on reduced disclosure requirements that are applicable to other public companies that are smaller reporting companies. Smaller reporting companies are able to provide simplified executive compensation disclosure and have certain other reduced disclosure obligations, including, among other things, being required to provide only two years of audited financial statements and not being required to provide supplemental financial information or risk factors. Despite status effectiveness at December 31, 2024, due to requalification we have been able to rely on these reduced requirements since June 30, 2024. We cannot predict whether investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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 February 28, 2025, we had outstanding 88,602,468 shares of common stock, which may be resold in the public market immediately without restriction, unless held by our affiliates. In connection with our January 2025 Offering, we and our officers and directors agreed with the underwriter, subject to certain exceptions, not to dispose of or hedge any of common stock or securities convertible into or exchangeable for shares of our common stock during the 60-day period after the date of the prospectus supplement, except with the prior written consent of the underwriter. Such agreements will expire on March 11, 2025.

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

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

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

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

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

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

- permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate (including the right to approve an acquisition or other change in our control);
- provide that the authorized number of directors may be changed only by resolution of the board of directors;
- provide that our board of directors or any individual director may only be removed with cause and the affirmative vote of the holders of at least 66-2/3% of the voting power of all of our then-outstanding common stock;
- provide that all vacancies, including newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- divide our board of directors into three classes;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner and also specify requirements as to the form and content of a stockholder's notice;
- do not provide for cumulative voting rights (therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose);
- provide that special meetings of our stockholders may be called only by the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors; and
- provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for the following types of actions or proceedings under state, statutory and common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (iii) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws; (iv) any action or proceeding to interpret, apply, enforce or determine the validity of our certificate of incorporation or our bylaws; (v) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers or other employees governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction

over the indispensable parties named as defendants; provided these provisions of our certificate of incorporation and bylaws will not apply to suits brought to enforce a duty or liability created by the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction; and provided that, unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the U.S. shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act of 1933, as amended, or the Securities Act.

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

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

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

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

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

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

Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. In addition, any testing by us or our independent registered public accounting firm conducted in connection with Section 404 may reveal deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock. Internal control deficiencies could also result in a restatement of our financial results in the future. We could become subject to stockholder or other third-party litigation, as well as investigations by the SEC, the Nasdaq Global Select Market, or other regulatory authorities, which could require additional financial and management resources and could result in fines, trading suspensions, payment of damages or other remedies. Further, any delay in compliance with the auditor attestation provisions of Section 404 could subject us to a variety of administrative sanctions, including ineligibility for short-form resale registration, action by the SEC and the suspension or delisting of our common stock, which could reduce the trading price of our common stock and could harm our business.

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

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

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

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

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

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

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

Pursuant to our 2022 Stock Option and Incentive Plan, or the 2022 Plan, our management is authorized to grant stock options to our employees, directors and consultants. Additionally, the number of shares of our common stock reserved for issuance under our 2022 Plan will automatically increase on January 1 of each year, beginning on January 1, 2023 and continuing through and including January 1, 2032, by 5.0% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. In addition, pursuant to our ESPP, the number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, beginning on January 1, 2023 (through January 1, 2032), by the lesser of (i) 1.0% of the total number of shares

of our common stock outstanding on the last day of the calendar month before the date of the automatic increase, and (ii) 1,210,000 shares; provided that before the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

General Risk Factors

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

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

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

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

We, our collaborators, our CROs, third-party logistics providers, distributors and other contractors and consultants utilize IT systems and networks to process, transmit and store electronic information in connection with our business activities. As use of digital technologies has increased, cyber incidents, including third parties gaining access to employee accounts using stolen or inferred credentials, ransomware, computer malware, viruses, denial-of-service, social engineering, spamming or other means, insider threats, and deliberate attacks and attempts to gain unauthorized access to computer systems and networks, have generally been increasing in frequency and sophistication. Cyber-attacks also could include phishing attempts or e-mail fraud to, for example, cause payments or information to be transmitted to an unintended recipient. Like other companies in our industry, we and some of our third-party collaborators have in the past and may in the future experience cyber security attacks. These threats pose a risk to the security of our, our collaborators', our CROs', third-party logistics providers', distributors' and other contractors' and consultants' systems and networks, and the confidentiality, availability and integrity of our data. There can be no assurance that we will be successful in preventing cyber-attacks or successfully mitigating their effects. Similarly, there can be no assurance that our collaborators, CROs, third-party logistics providers, distributors and other contractors and consultants will be successful in protecting our clinical and other data that is stored on their systems or to which they have access. Any cyber-attack, data breach, security incident or destruction, misuse, or loss of data could require us to notify impacted stakeholders (including affected individuals, regulators, and investors), result in a violation of applicable U.S. and international privacy, data protection and other laws, and subject us to litigation and governmental investigations and proceedings by federal, state and local regulatory entities in the U.S. and by international regulatory entities, resulting in exposure to material civil and/or criminal liability. Further, our cybersecurity insurance, 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 avexitide, 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 or incidents and may incur reputational harm and significant additional expense, including to implement further data protection or remedial measures, from fines and penalties or other liability, and from loss of existing and future business. Further, our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our privacy and data security obligations.

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, 2024, we had U.S. federal net operating loss, or NOL, carryforwards of $262.7 million that carry forward indefinitely. The amount of annual utilization of these NOL carryforwards may be limited based on provisions of the Tax Cuts and Jobs Act of 2017, or TCJA. As of December 31, 2024, we also had U.S. federal research and development tax credit carryforwards of $13.4 million and we have additionally recorded deferred tax assets for U.S. state NOL and research and development tax credit carryforwards of $11.2 million. These U.S. federal research and development tax credit and U.S. state carryforwards could begin to expire if unused in 2042 and 2035, respectively. Utilization of all NOL and research and development tax credit carryforwards is conditioned upon us generating U.S. federal and state taxable income.

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

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

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 pharmaceutical companies have experienced significant stock price volatility in recent years. Such litigation could result in substantial costs and a diversion of management's attention and resources, which could harm our business. For further information, see "Item 3. - Legal Proceedings."

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

If we fail to satisfy the continued listing requirements of the Nasdaq Global Select Market, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase our common stock when you wish to do so. In the event of a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping below the minimum bid price requirement or prevent future non-compliance with the listing requirements of the Nasdaq Global Select Market.

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

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

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity.

Risk Management and Strategy

We recognize that cybersecurity threats have been increasing in number and severity in the general marketplace and in our industry. In an effort to address the threat landscape, we maintain a cybersecurity risk management strategy that is designed to identify, assess, manage, and address cybersecurity threats that may have a material impact on our business. Our cybersecurity risk management strategy includes various policies and components, including cybersecurity assessments, an incident response plan, evaluation of the security practices of our key vendors, and cybersecurity awareness training for our staff.

We engage a third-party to conduct a cybersecurity risk assessment on an annual basis, which is informed by the National Institute of Standards and Technology, or NIST Cybersecurity Framework. We have established a process for our IT security team to track and quantify known IT security risks and our remediation efforts through a cybersecurity risk register. The IT security team meets periodically to review and update the cybersecurity risk register based on feedback across the organization and the findings contained in our NIST-informed annual cybersecurity risk assessment. The IT security team reports on findings on at least an annual basis to the executive leadership team and the board of directors.

We have established a process to review and assess vendors' security posture and practices prior to their onboarding. Vendors that access, store or process our data are required to respond to a cybersecurity questionnaire and provide applicable security audit reports and certifications. Our process also includes contractual requirements to maintain data protection safeguards for vendors that process data on our behalf.

We maintain a security awareness training program for employees, which is provided through digital microlearning assignments. We also provide additional mandatory trainings, including phishing training, throughout the year.

We maintain a Written Information Security Program, or WISP, that defines our organization's cybersecurity policies and procedures. This covers all aspects of cybersecurity, including but not limited to risk management, third party security assessments, security awareness training, acceptable use, endpoint security, patch management, log management, backup and recovery.

We face a number of cybersecurity risks in connection with our business. Although such risks have not materially affected, and we do not believe they are reasonably likely to materially affect, our business strategy, results of operations or financial condition, to date, we have, from time to time, experienced threats to and security incidents related to our and our third-party vendors' information systems. For more information about the cybersecurity risks we face, see the risk factor entitled "Cyber-attacks or other failures in our telecommunications or IT systems, or those of our collaborators, CROs, third-party logistics providers, distributors or other contractors or consultants, could result in information theft, data corruption and significant disruption of our business operations" in Item 1A- Risk Factors.

Governance of Cybersecurity Risks

Our board of directors is responsible for the general oversight of cybersecurity risks and is informed of key updates to our cybersecurity processes by relevant members of our executive leadership team on at least an annual basis.

Our executive leadership team meets with our Head of Global Information Technology, along with other members of our IT security team as needed, to discuss cybersecurity matters, such as the emerging cybersecurity threat landscape, significant developments to our cybersecurity processes, and our cybersecurity risk assessments. Senior management is thus kept abreast of the cybersecurity posture and potential risks facing our company. Our cybersecurity incident response process is designed to proactively triage, contain, investigate, mitigate and correct all incidents at the direction of the Head of Global Information Technology. Critical incidents are assessed for materiality, and escalated to the executive leadership team for awareness, direction and approval as needed. Furthermore, significant cybersecurity matters, and strategic risk management decisions are escalated to the Board of Directors, as needed, to provide oversight and guidance on critical cybersecurity issues.

Our IT security team, led by the Head of Global Information Security, Governance and Architecture ("Head of Global ISGA"), is responsible for managing and directing the day-to-day information security strategy of the organization, including oversight of our cybersecurity tools, controls and strategies to protect organization assets, networks and data. The Head of Global ISGA reports to our Head of Global Information Technology. The Head of Global ISGA routinely reports on cybersecurity risks, projects, and initiatives to the Head of Global Information Technology, who regularly reports to executive management and the audit committee on these matters as described above.

The Head of Global ISGA maintains a Certified Information Systems Security Professionals, or CISSP, certification and has more than two decades of IT security management experience. The IT security team is supported by external vendors that provide managed services for network support, security operations and other IT areas as needed. Our IT security team also meets regularly with our Global Privacy Committee, which oversees our Enterprise Data Protection Program, to coordinate on cybersecurity initiatives and strategy related to protection of personal data.

Item 2. Properties.

Details of our principal properties as of December 31, 2024, are provided below:

Property Description	Location	Square Footage	Property Interest	Initial Lease Term End Date
Office space	Cambridge, Massachusetts	8,850	Leased	October 2026
Office space	Cambridge, Massachusetts	24,400	Leased	July 2025
Office space	Cambridge, Massachusetts	15,267	Leased	December 2030

We believe that our facilities are adequate for our current needs and that suitable additional or substitute space would be available if needed.

Item 3. Legal Proceedings.

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

On August 12, 2024, the case was transferred from the U.S. District Court for the Southern District of New York to the U.S. District Court for the District of Massachusetts, or the Court, and assigned docket number 1:24-CV-12068. Following the transfer, on September 6, 2024, defendants moved to dismiss the Shih Complaint. Plaintiff filed his opposition to defendants' motion to dismiss on October 21, 2024 and defendants filed their reply brief in further support of their motion to dismiss on November 20, 2024.

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

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

Item 4. Mine Safety Disclosures.

Not Applicable.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock began trading on The Nasdaq Global Select Market on January 7, 2022, under the symbol "AMLX." Prior to that time, there was no public market for our common stock.

Holders of Record

As of February 28, 2025, we had approximately 17 holders of record of our common stock. Certain shares are held in "street" name and accordingly, the number of beneficial owners of such shares is not known or included in the foregoing number. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividend Policy

We have never declared or paid a dividend on our common stock, and we do not anticipate declaring or paying dividends on our common stock in the foreseeable future. We currently intend to retain our future earnings, if any, to fund the development and growth of our business.

Securities Authorized for Issuance under Equity Compensation Plans

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

We did not purchase any of our registered equity during the period covered by this Annual Report.

Unregistered Sales of Securities

During the year ended December 31, 2024, we did not issue or sell any unregistered securities.

Item 6. [Reserved]

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

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

This discussion and other parts of this Annual 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 the "Risk Factors" section of this Annual Report, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage pharmaceutical company with a mission to develop novel therapies for communities with high unmet medical needs. We have preclinical and clinical development programs underway in neurodegenerative diseases and endocrine conditions. We are advancing a pipeline in which we have matched investigational therapies with diseases where they can make the greatest impact, based on well-defined mechanistic rationale, clear clinical outcomes and biomarkers, and rigorous preclinical data, agnostic of modality. We are currently developing three investigational therapies for potential impact across several diseases: avexitide in PBH and congenital HI, AMX0035 in Wolfram syndrome and PSP, and AMX0114 in ALS.

As of December 31, 2024, we had cash, cash equivalents and marketable securities of $176.5 million. In January 2025, we received net proceeds of $65.5 million from the January 2025 Offering. We believe our existing cash, cash equivalents and marketable securities as of December 31, 2024, along with the proceeds from the January 2025 Offering, will be sufficient to meet our anticipated operating and capital expenditure requirements through 2026. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See "Liquidity and Capital Resources" below.

Components of Our Results of Operations

Product Revenue, Net

Product revenue, net recognized during the years ended December 31, 2024 and 2023 relates to units of ALBRIOZA and RELYVRIO sold in Canada and the U.S., respectively. In April 2024, we announced we had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ (sodium phenylbutyrate and taurursodiol [also known as ursodoxicoltaurine]; also known as AMX0035) for the treatment of ALS and remove the product from the market in the U.S. and Canada, or the "RELYVRIO®/ALBRIOZA™ Discontinuation. This decision was informed by topline results from the global Phase 3 PHOENIX trial, which failed to meet its prespecified primary and secondary endpoints, engagement with regulatory authorities, and discussions with the ALS community. As a result, we do not expect to generate revenue from the sale of RELYVRIO®/ALBRIOZA™ in future periods.

As of April 4, 2024, RELYVRIO/ALBRIOZA was no longer available for new patients. Patients who were currently on therapy in the U.S. and Canada who, in consultation with their physician, wished to stay on treatment had the option to be transitioned to a free drug program. Patients and their physicians were informed that final shipments of free drug were made to allow treatment through early 2025. The NDA is now on the Discontinued Drug Product List of the Orange Book and we have filed a formal request to withdraw the NDA. We have completed the collection of data on survival and we will continue to share any learnings from PHOENIX to help inform future ALS research. We wound down the Open Label Extension as planned.

Operating Expenses

Cost of Sales

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

Acquired In-process Research and Development Expenses

Acquired in-process research and development, or IPR&D, expenses relate to upfront or other payments pursuant to our business development transactions, including the Eiger Acquisition (as defined below).

Research and Development Expenses

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

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

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

Certain of our indirect research and development expenses are not tracked on an indication-by-indication basis. 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 avexitide and AMX0035 in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, such as AMX0114, primarily due to the increased size and duration of later-stage clinical trials and related product manufacturing expenses. Despite a decline in research and development expenses in 2024 compared to 2023, we expect that our research and development expenses will increase in connection with our planned clinical development activities in the near term and in the future. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the clinical development of avexitide, 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 studies of our current or future product candidates; and
- the number of product candidates we are developing.

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

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical trials for separate indications we decide to pursue;
- raising additional funds, if necessary;
- the progress of the development efforts of parties with whom we may enter into collaboration arrangements;
- our ability to maintain our current development activities and to establish new ones;
- our ability to establish new licensing or collaboration arrangements;

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

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

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

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

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

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

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

•competition with other products; and

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

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

Selling, General and Administrative Expenses

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

Restructuring Expenses

Restructuring expenses consists primarily of employee severance and termination benefits, contract termination costs, impairment of long-lived assets and other costs. Such costs are based on estimates of fair value in the period liabilities are incurred. We evaluate and adjust these costs as appropriate for changes in circumstances as additional information becomes available.

Income Taxes

We have historically not incurred significant income taxes. We continue to maintain a full valuation allowance against all of our deferred tax assets based on management's evaluation of all available evidence, including our history of incurring significant losses from operations. As a result, we don't expect to incur material income taxes for the foreseeable future.

Results of Operations

Comparison of the Years Ended December 31, 2024 and 2023

The following table summarizes our results of operations for the years ended December 31, 2024 and 2023:

	Year Ended December 31,			
	2024	2023	$ Change	% Change
	(in thousands)			
Product revenue, net	$ 87,371	$ 380,786	$ (293,415)	(77)%
Operating expenses:				
Cost of sales	5,953	25,441	(19,488)	(77)%
Cost of sales - inventory impairment and loss on firm purchase commitments	118,680	—	118,680	*NM
Acquired in-process research and development	36,203	—	36,203	*NM
Research and development	104,084	128,187	(24,103)	(19)%
Selling, general and administrative	114,331	188,356	(74,025)	(39)%
Restructuring expenses	22,851	—	22,851	*NM
Total operating expenses	402,102	341,984	60,118	18%
(Loss) income from operations	(314,731)	38,802	(353,533)	(911)%
Other income, net:				
Interest income	13,809	16,155	(2,346)	(15)%
Other expense, net	(1,214)	(660)	(554)	84%
Total other income, net	12,595	15,495	(2,900)	(19)%
(Loss) income before income taxes	(302,136)	54,297	(356,433)	(656)%
(Benefit) provision for income taxes	(393)	5,026	(5,419)	(108)%
Net (loss) income	$ (301,743)	$ 49,271	$ (351,014)	(712)%

* NM - not meaningful

Product revenue, net

Product revenue, net was $87.4 million for the year ended December 31, 2024, compared to $380.8 million for the year ended December 31, 2023. Product revenue, net was related to units of RELYVRIO and ALBRIOZA sold in the U.S. and Canada, respectively, prior to the RELYVRIO®/ALBRIOZA™ Discontinuation.

Cost of sales

Cost of sales were $124.6 million for the year ended December 31, 2024, compared to $25.4 million for the year ended December 31, 2023. Cost of sales consisted of costs to procure, manufacture and distribute our marketed products, RELYVRIO and ALBRIOZA. As a result of the RELYVRIO®/ALBRIOZA™ Discontinuation, we recorded approximately $118.7 million of charges associated with the write-down of inventory and losses on firm purchase commitments for the year ended December 31, 2024.

Acquired In-process Research and Development Expenses

On July 9, 2024, we completed the acquisition of substantially all the assets and interests in the development, manufacture and commercialization of avexitide, an investigational, first-in-class GLP-1 receptor antagonist, from Eiger, or the Seller, or the Eiger Acquisition. During the year ended December 31, 2024, we recorded a charge of approximately $36.2 million associated with the acquired in-process research and development assets of avexitide with no alternative future use.

Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2024 and 2023:

	Year Ended December 31,			
	2024	2023	$ Change	% Change
	(in thousands)			
Direct research and development expenses by program:				
AMX0035 - ALS	$ 36,727	$ 60,843	$ (24,116)	(40)%
AMX0035 - PSP	16,917	6,404	10,513	164%
Avexitide	2,766	—	2,766	*NM
Other programs	8,698	13,870	(5,172)	(37)%
Total direct research and development expenses by program	65,108	81,117	(16,009)	(20)%
Personnel-related research and development	38,976	47,070	(8,094)	(17)%
	$ 104,084	$ 128,187	$ (24,103)	(19)%

* NM - not meaningful

Research and development expenses were $104.1 million for the year ended December 31, 2024, compared to $128.2 million for the year ended December 31, 2023. The decrease of $24.1 million was primarily due to a $24.1 million decrease in spending on AMX0035 for the treatment of ALS following topline data from the PHOENIX trial in April 2024, an $8.1 million decrease in payroll and personnel-related costs due to a decrease in the number of employees following the completion of our Restructuring Plan, and a $5.2 million decrease in other costs due to an decrease in preclinical development activities.

The decrease in research and development expenses was offset by a $10.5 million increase in spending on AMX0035 for the treatment of PSP, and a $2.8 million increase in expenses for the development of avexitide following the Eiger Acquisition. The increase in spending on AMX0035 for the treatment of PSP was primarily related to costs to support the continuation of the ORION Phase 2b/3 global clinical trial.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $114.3 million for the year ended December 31, 2024 compared to $188.4 million for the year ended December 31, 2023. The decrease was primarily due to a decrease of $39.5 million in payroll and personnel-related costs, $25.5 million in consulting and professional services, and $9.1 million in other expenses. The decrease in payroll and personnel-related costs was primarily related to a decrease in the number of employees as a result of the Restructuring Plan. The decrease in consulting and professional services was primarily due to a decrease in commercial sales and marketing activity as a result of the RELYVRIO®/ALBRIOZA™ Discontinuation. The decrease in other expenses is primarily due to a decrease in charitable contributions.

Restructuring Expenses

In April 2024, we announced a restructuring to focus our financial resources on upcoming clinical milestones. Under the restructuring, we reduced our workforce by approximately 70% and decreased external financial commitments outside of our priority areas. Restructuring expenses were approximately $22.9 million for the year ended December 31, 2024 which includes employee severance and termination benefits of approximately $21.9 million, contract termination costs, impairment of long-lived assets and other costs of $1.0 million. We completed the Restructuring Plan in 2024.

Liquidity and Capital Resources

Sources of Liquidity

In the second half of 2022, we began generating revenue from the sale of our approved drug product RELYVRIO, known as ALBRIOZA in Canada. In April 2024, we announced the RELYVRIO®/ALBRIOZA™ Discontinuation. We also announced the Restructuring Plan, which was designed to focus our resources on key clinical and preclinical programs and included a reduction in force which reduced our workforce by approximately 70% and decreased external financial commitments outside of our priority areas. We completed the Restructuring Plan in the second half of 2024.

In January 2025, we entered into an underwriting agreement with Leerink Partners LLC, as representative of the several underwriters named therein, relating to the issuance and sale of an aggregate of 19,714,285 shares of our common stock, which includes the exercise in full by the underwriters of their option to purchase an additional 2,571,428 shares, or the January 2025 Offering. The offering price per share was $3.50. The January 2025 Offering resulted in estimated proceeds of approximately $65.5 million, net of underwriting discounts and estimated offering expenses (see Note 18 Subsequent events).

As of December 31, 2024, we had cash, cash equivalents and marketable securities of $176.5 million and an accumulated deficit of $606.7 million. We believe our existing cash, cash equivalents and marketable securities as of December 31, 2024, along with the proceeds from the January 2025 Offering, will be sufficient to meet our anticipated operating and capital expenditure requirements through 2026. 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.

Since inception, we have devoted substantially all of our efforts to research and development, pre-commercialization and commercialization activities, including recruiting management and technical staff, raising capital, producing materials for preclinical studies and clinical trials, and building infrastructure to support such activities. As of December 31, 2024, we have funded our operations primarily through public offerings of our common stock, private sales of preferred stock, convertible notes, and through revenue from sales of RELYVRIO and ALBRIOZA in the U.S. and Canada, respectively between July 2022 and April 2024.

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

Capital Resources

Despite the decline in research and development and general administrative expenses in 2024 as compared to 2023, we expect our expenses to increase in connection with our ongoing activities, particularly as we advance the preclinical activities, manufacturing and clinical trials of avexitide, AMX0035 and any other current or future product candidates or acquire or in-license additional product candidates or products. We may also incur expenses related to business development activities, such as in-licensing or acquisition of product candidates. In addition, we expect to incur additional costs associated with operating as a public company, including significant legal, accounting, investor relations and other expenses. We expect to incur significant expenses as we:

- continue our research and development efforts of avexitide in PBH, or any other indications, and conduct clinical trials of avexitide;
- continue our research and development efforts of AMX0035, including our ongoing Phase 2b/3 trial of AMX0035 in PSP and our ongoing Phase 2 trial of AMX0035 for the treatment of Wolfram syndrome;
- continue to develop AMX0114, our antisense oligonucleotide, for the treatment of people living ALS;
- pursue INDs of AMX0035 for additional indications;
- conduct preclinical studies and clinical trials for AMX0035 for additional indications and for potential future product candidates;
- seek to identify and develop, acquire or in-license additional product candidates;

- experience any delays or encounter any issues with any of the above, including but not limited to failed studies, complex results, safety issues, or other regulatory challenges;
- develop the necessary processes, controls and manufacturing data to obtain marketing approval for current or future product candidates and to support manufacturing on a commercial scale;
- seek regulatory approvals for any current or future product candidates that successfully complete clinical trials, if any;
- incur expenses in preparation for commercialization for any approved product candidates related to product sales, marketing, manufacturing, and distribution;
- hire and retain additional personnel, such as preclinical, clinical, quality assurance, regulatory affairs, manufacturing, distribution, legal, compliance, finance, general and administrative, commercial and scientific personnel; and
- develop, maintain, expand and protect our intellectual property portfolio.

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

- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical and clinical development for avexitide, AMX0035 and any future product candidates;
- the costs, timing and outcome of any future commercialization activities, including manufacturing, marketing, sales and distribution costs;
- the costs, timing and outcome of regulatory review of avexitide, AMX0035 and any future product candidates;
- our ability to establish and maintain collaborations, marketing, distribution and license agreements on favorable terms, if at all;
- our ability to enroll clinical trials in a timely manner and to quickly resolve any delays or clinical holds that may be imposed on our development activities;
- timing delays with respect to preclinical and clinical development of avexitide, AMX0035 and any future product candidates, including as result of any future outbreak of any highly infectious or contagious diseases;
- costs associated with identifying and developing, acquiring or in-licensing additional product candidates or products;
- the costs of any future expansion of our facilities to accommodate our potential growth in personnel, and the costs of such additional personnel;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire technologies or other assets;
- the sales price and availability of adequate third-party coverage and reimbursement for any future product candidates, if and when approved;
- the costs of current and potential legal proceedings that may not be covered by our insurance; and
- the costs of operating as a public company.

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

Cash Flows

Comparison of the Years Ended December 31, 2024 and 2023

The following table summarizes our sources and uses of cash for the years ended December 31, 2024 and 2023:

	Year Ended December 31,			
	2024	2023	$ Change	% Change
	(in thousands)			
Net cash (used in) provided by operating activities	$ (167,647)	$ 11,919	$ (179,566)	(1,507)%
Net cash provided by investing activities	75,654	92,053	(16,399)	(18)%
Net cash provided by financing activities	348	3,543	(3,195)	(90)%
Effect of exchange rate changes on cash, cash equivalents and restricted cash equivalents	(438)	160	(598)	(374)%
Net (decrease) increase in cash, cash equivalents and restricted cash equivalents	$ (92,083)	$ 107,675	$ (199,758)	(186)%

Operating Activities

During the year ended December 31, 2024, operating activities used $167.6 million of cash, primarily resulting from our net loss of $301.7 million, offset by non-cash items totaling $179.9 million including $118.7 million of inventory impairment and loss on firm purchase commitments, $33.0 million of non-cash stock-based compensation expense, $9.9 million in accretion of discounts on investments and $36.2 million of acquired IPR&D assets, which are classified as investing activities.

Changes in working capital totaled $45.8 million, primarily consisting of a $59.8 million decrease in accrued expenses, a $19.1 million decrease in accounts payable, a $9.3 million decrease in inventories and partially offset by a $39.6 million decrease in accounts receivable, net.

During the year ended December 31, 2023, operating activities provided $11.9 million of cash, primarily resulting from our net income of $49.3 million, non-cash stock-based compensation expense of $37.2 million and $1.1 million of depreciation expense, offset by an increase of $65.7 million in net cash used in our operating assets and liabilities and net amortization of premiums and discounts on investments of $9.9 million.

Net cash used in our operating assets and liabilities in 2023 primarily consisted of a $21.6 million increase in accrued expenses, a $15.9 million increase in accounts payable and a $1.8 million decrease in operating lease right-of-use assets. This was offset by a $73.1 million increase in inventories, a $24.7 million increase in accounts receivable, a $4.8 million increase in prepaid expenses and other current assets and a $2.0 million decrease in operating lease liabilities.

Investing Activities

During the year ended December 31, 2024, net cash provided by investing activities was $75.7 million resulting from $344.0 million of investments that matured during the period offset by $232.0 million in purchases of marketable securities and a $36.2 million cash outflow to acquire IPR&D assets related to the Eiger Acquisition.

During the year ended December 31, 2023, net cash provided by investing activities was $92.1 million resulting from $394.1 million of investments matured during the period offset by $300.8 million in purchases of marketable securities and $1.2 million in purchases of property and equipment.

Financing Activities

During the year ended December 31, 2024, net cash provided by financing activities was $0.3 million. This amount consisted of $2.1 million of proceeds from exercises of stock options, offset by $1.8 million of withholding taxes paid on stock-based awards.

During the year ended December 31, 2023, net cash provided by financing activities was $3.5 million. This amount consisted of $7.0 million of proceeds from exercises of stock options, offset by $3.3 million of withholding taxes paid on stock-based awards and $0.1 million in payments of deferred offering costs.

Contractual Obligations and Commitments

We enter into agreements in the normal course of business with contract manufacturing organizations for raw material purchases and manufacturing services. As of December 31, 2024, the amounts committed under these agreements are not material.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with U.S. GAAP. 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.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements appearing at the end of this Annual Report, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Revenue Recognition

Prior to the RELYVRIO®/ALBRIOZA™ Discontinuation, our accounting policy for revenue recognition had a substantial impact on reported results and relied on certain estimates. Specifically, revenue was reduced by variable consideration related to certain gross-to-net, or GTN. These GTN adjustments involve significant estimates and judgment, considering historical experience, payer channel mix (e.g., Medicare or Medicaid), current contract prices under applicable programs, unbilled claims and processing time lags and inventory levels in the distribution channel. Estimates were assessed each period and adjusted as required to revise information or actual experience. To date, actual GTN activity has not differed materially from our estimates. Because of the time elapsed since the RELYVRIO®/ALBRIOZA™ Discontinuation the remaining estimates and judgments related to revenue recognition are not considered significant as of December 31, 2024.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating

with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments if necessary.

The estimate of accrued research and development expense is dependent, in part, upon the receipt of timely and accurate reporting from CROs, CMOs and other third-party service providers. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with preclinical development activities;
- CROs and investigative sites in connection with preclinical studies and clinical trials; and
- CMOs in connection with drug substance and drug product formulation of preclinical study and clinical trial materials.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CMOs and CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expenses accordingly. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk.

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, and are not required to provide the information required under this item.

Item 8. Financial Statements and Supplementary Data.

Our consolidated financial statements, together with the reports of our independent registered public accounting firms, appear beginning on page F-1 of this Annual Report.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, are designed to ensure that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our co-Chief Executive Officers and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officers and our principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2024. Based on the evaluation of our disclosure controls and procedures as of December 31, 2024, our co-Chief Executive Officers and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed 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. Our internal control over financial reporting include policies and procedures that:

- pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect transactions relating to our business and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with U.S. GAAP, and that receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision and with the participation of the Company's Chief Executive Officers and the Company's Chief Financial Officer, we assessed the effectiveness of our internal control over financial reporting based on the criteria for effective internal control over financial reporting under the 2013 "Internal Control —Integrated Framework", issued by the

Committee of Sponsoring Organizations of the Treadway Commission, or COSO. Based on such assessment, our management concluded that we maintained effective internal control over financial reporting as of December 31, 2024.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during the quarter ended December 31, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Amylyx Pharmaceuticals, Inc.

Opinion on Internal Control over Financial Reporting

We have audited the internal control over financial reporting of Amylyx Pharmaceuticals, Inc. and subsidiaries (the "Company") as of December 31, 2024, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2024, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by COSO.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated financial statements as of and for the year ended December 31, 2024, of the Company and our report dated March 4, 2025, expressed an unqualified opinion on those financial statements.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Annual Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control over Financial Reporting

A company's internal control over financial reporting is a process designed 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. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
March 4, 2025

Item 9B. Other Information.

(a)

None.

(b)

Rule 10b5-1 Trading Arrangements

During the three months ended December 31, 2024, no officers or directors of the Company (as defined in Rule 16a-1(f)) adopted, modified or terminated a "Rule 10b5-1 trading arrangement" or a "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(c) of Regulation S-K of the Exchange Act, which are intended to satisfy the affirmative defense conditions of Rule 10b5-1(c).

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this Item 10 will be included in the Proposal No. 1, Corporate Governance and Executive Officers section of our definitive proxy statement to be filed with the SEC with respect to our 2025 Annual Meeting of Stockholders and is incorporated herein by reference.

Item 11. Executive Compensation.

The information required by this Item 11 will be included in the Executive Compensation and Director Compensation sections (excluding the information under the heading "Pay Versus Performance") of our definitive proxy statement to be filed with the SEC with respect to our 2025 Annual Meeting of Stockholders and is incorporated herein by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this Item 12 will be included in the Security Ownership of Certain Beneficial Owners and Management sections of our definitive proxy statement to be filed with the SEC with respect to our 2025 Annual Meeting of Stockholders and is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this Item 13 will be included in the Certain Relationships and Related Party Transactions and Corporate Governance sections of our definitive proxy statement to be filed with the SEC with respect to our 2025 Annual Meeting of Stockholders and is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services.

Our independent public accounting firm is Deloitte & Touche LLP, Boston, Massachusetts, PCAOB Auditor ID: 34.

The information required by this Item 14 will be included in the Proposal No. 2 section of our definitive proxy statement to be filed with the SEC with respect to our 2025 Annual Meeting of Stockholders and is incorporated herein by reference.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

a) Financial Statements

For a list of the consolidated financial statements included herein, see Index to the Consolidated Financial Statements on page F-1 of this Annual Report, which is incorporated into this Item by reference.

b) Exhibits

Exhibit Number	Description
2.1†	Asset Purchase Agreement by and between the Company and Eiger Biopharmaceuticals, Inc., dated June 21, 2024 (Incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on June 21, 2024).
3.1	Fourth Amended and Restated Certificate of Incorporation of Amylyx Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on January 11, 2022).
3.2	Second Amended and Restated Bylaws of Amylyx Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on January 11, 2022).
4.1	Specimen Common Stock Certificate (Incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).
4.2	Second Amended and Restated Investors' Rights Agreement, dated as of July 1, 2021, among the Registrant and the parties thereto (Incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1 (File No. 333-261703) filed with the Securities and Exchange Commission on December 16, 2021).
4.3	Description of Securities (Incorporated by reference to Exhibit 4.3 to the Registrant's Form 10-K filed with the Securities and Exchange Commission on March 31, 2022).
10.1#	2015 Stock Option and Incentive Plan, and form of award agreements thereunder (Incorporated by reference to Exhibit 10.1 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).
10.2#	2022 Stock Option and Incentive Plan, and form of award agreements thereunder (Incorporated by reference to Exhibit 10.2 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).
10.3#	Amended and Restated Non-Employee Director Compensation Policy (Incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 9, 2024).
10.4#	Executive Cash Incentive Bonus Plan (Incorporated by reference to Exhibit 10.4 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).
10.5#	2022 Employee Stock Purchase Plan (Incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).
10.6#	Lease Agreement, dated as of October 23, 2018, as amended, by and between the Registrant and Bullfinch Square Limited Partnership (Incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1 (File No. 333-261703) filed with the Securities and Exchange Commission on December 16, 2021).
10.7#	Form of Employment Agreement, between the Registrant and Josh Cohen (Incorporated by reference to Exhibit 10.7 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).
10.8#	Form of Employment Agreement, between the Registrant and Justin Klee (Incorporated by reference to Exhibit 10.8 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).

10.9#	Form of Employment Agreement, between the Registrant and James Frates (Incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on Form S-1/A (File No. 333-261703) filed with the Securities and Exchange Commission on January 3, 2022).
10.10#	Form of Employment Agreement, between the Registrant and Gina Mazzariello (Incorporated by reference to Exhibit 10.18 to the Registrant's Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 13, 2023).
10.11#*	Form of Employment Agreement, between the Registrant and Camille Bedrosian.
10.12#	Form of Officer Indemnification Agreement (Incorporated by reference to Exhibit 10.13 to the Registrant's Registration Statement on Form S-1 (File No. 333-261703) filed with the Securities and Exchange Commission on December 16, 2021).
10.13†	Master Manufacturing Services Agreement, dated as of November 12, 2019, by and between the Registrant and Patheon Inc. (Incorporated by reference to Exhibit 10.14 to the Registrant's Registration Statement on Form S-1 (File No. 333-261703) filed with the Securities and Exchange Commission on December 16, 2021).
10.14†	First Amendment, dated as of January 18, 2021, to Product Agreement, dated as of November 12, 2019, pursuant to the Master Manufacturing Services Agreement, dated as of November 12, 2019, by and between the Registrant and Patheon Inc. (Incorporated by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 11, 2023).
10.15†	Second Amendment, dated as of March 20, 2023, to Product Agreement, dated as of November 12, 2019, as amended by Amendment No. 1, dated as of January 18, 2021, pursuant to the Master Manufacturing Services Agreement, dated as of November 12, 2019, by and between the Registrant and Patheon Inc. (Incorporated by reference to Exhibit 10.3 to the Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 11, 2023).
10.16†	Supply Agreement, dated as of October 29, 2019, by and between the Registrant and CU Chemie Uetikon GmbH (Incorporated by reference to Exhibit 10.15 to the Registrant's Registration Statement on Form S-1 (File No. 333-261703) filed with the Securities and Exchange Commission on December 16, 2021).
10.17†	First Amendment, effective as of January 1, 2023, to the Supply Agreement, dated as of October 29, 2019, by and between the Registrant and CU Chemie Uetikon GmbH (Incorporated by reference to Exhibit 10.4 to the Registrant's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 11, 2023).
10.18†	Research, Development and Supply Agreement, dated as of December 9, 2019, and Deed of Amendment, dated as of July 26, 2021, by and between the Registrant and ICE S.p.A. (formerly Prodotti Chimici e Alimentari S.p.A.), as amended (Incorporated by reference to Exhibit 10.16 to the Registrant's Registration Statement on Form S-1 (File No. 333-261703) filed with the Securities and Exchange Commission on December 16, 2021).
10.19†	Commercial Supply Agreement, dated as of August 8, 2023, by and between the Registrant and ICE S.p.A. (formerly Prodotti Chimici e Alimentari S.p.A.) (Incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on form 10-Q filed with the Securities and Exchange Commission on August 10, 2023).
19.1*	Amylyx Pharmaceuticals, Inc. Insider Trading Policy.
21.1*	List of Subsidiaries of Registrant.
23.1*	Consent of Deloitte & Touche LLP, independent registered public accounting firm.
31.1*	Certification of Co-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 Co-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 Co-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 Co-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.
97.1*	Compensation Recovery Policy.

101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.
101.SCH*	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith.

+ Furnished 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.

† Portions of this exhibit (indicated by asterisks) have been omitted in accordance with Item 601(b)(10) of Regulation S-K.

c)Financial Statement Schedules

No financial statements have been submitted because they are not required or are not applicable or because the information required is included in the consolidated financial statements or the notes thereto.

Item 16. Form 10-K Summary

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this Report to be signed on its behalf by the undersigned, thereunto duly authorized**.**

AMYLYX PHARMACEUTICALS, INC.

Date: March 4, 2025	By:	/s/ Joshua B. Cohen **Joshua B. Cohen** **Co-Chief Executive Officer**
Date: March 4, 2025	By:	/s/ Justin B. Klee **Justin B. Klee** **Co-Chief Executive Officer**

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

Name	Title	Date
/s/ Joshua B. Cohen **Joshua B. Cohen**	Co-Chief Executive Officer and Director (Principal Executive Officer)	March 4, 2025
/s/ Justin B. Klee **Justin B. Klee**	Co-Chief Executive Officer and Director (Principal Executive Officer)	March 4, 2025
/s/ James M. Frates **James M. Frates**	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 4, 2025
/s/ George Mclean Milne Jr. **George Mclean Milne Jr. Ph.D.**	Director	March 4, 2025
/s/ Paul Fonteyne **Paul Fonteyne, M.S., M.B.A.**	Director	March 4, 2025
/s/ Daphne Quimi **Daphne Quimi**	Director	March 4, 2025
/s/ Karen Firestone **Karen Firestone**	Director	March 4, 2025
/s/ Bernhardt Zeiher **Bernhardt Zeiher, MD.**	Director	March 4, 2025

Amylyx Pharmaceuticals, Inc.
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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Amylyx Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Amylyx Pharmaceuticals, Inc. and subsidiaries (the "Company") as of December 31, 2024 and 2023, the related consolidated statements of operations, comprehensive income (loss), stockholders' equity, and cash flows, for each of the years then ended, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the years then ended, in conformity with accounting principles generally accepted in the United States of America.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2024, based on criteria established in *Internal Control — Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission and our report dated March 4, 2025, expressed an unqualified opinion on the Company's internal control over financial reporting.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

Critical audit matters are matters arising from the current-period audit of the financial statements that were communicated or required to be communicated to the audit committee and that (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. We determined that there are no critical audit matters.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
March 4, 2025

We have served as the Company's auditor since 2020.

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

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 77,391	$ 170,201
Marketable securities	99,110	201,161
Accounts receivable, net	447	40,050
Inventories	—	38,323
Prepaid expenses and other current assets	12,484	14,931
Total current assets	189,432	464,666
Property and equipment, net	961	2,686
Restricted cash equivalents	1,446	719
Operating lease right-of-use assets	1,771	3,725
Long-term inventories	—	44,957
Other assets	24	701
Total assets	$ 193,634	$ 517,454
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,939	$ 22,061
Accrued expenses	23,949	57,724
Operating lease liabilities, current portion	1,518	2,257
Total current liabilities	28,406	82,042
Operating lease liabilities, net of current portion	463	1,980
Total liabilities	28,869	84,022
Commitments and contingencies (Note 16)		
Stockholders' equity:		
Common stock, $0.0001 par value; 300,000,000 shares authorized; 68,629,738 and 67,707,432 shares issued and outstanding as of December 31, 2024 and 2023, respectively	7	7
Additional paid-in capital	771,542	738,177
Accumulated deficit	(606,692)	(304,949)
Accumulated other comprehensive (loss) income	(92)	197
Total stockholders' equity	164,765	433,432
Total liabilities and stockholders' equity	$ 193,634	$ 517,454

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

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

	Year Ended December 31,	
	2024	**2023**
Product revenue, net	$ 87,371	$ 380,786
Operating expenses:		
Cost of sales	5,953	25,441
Cost of sales - inventory impairment and loss on firm purchase commitments	118,680	—
Acquired in-process research and development	36,203	—
Research and development	104,084	128,187
Selling, general and administrative	114,331	188,356
Restructuring expenses	22,851	—
Total operating expenses	402,102	341,984
(Loss) income from operations	(314,731)	38,802
Other income, net:		
Interest income	13,809	16,155
Other expense, net	(1,214)	(660)
Total other income, net	12,595	15,495
(Loss) income before income taxes	(302,136)	54,297
(Benefit) provision for income taxes	(393)	5,026
Net (loss) income	$ (301,743)	$ 49,271
Net (loss) income per share		
Basic	$ (4.43)	$ 0.73
Diluted	$ (4.43)	$ 0.70
Weighted-average shares used in computing net (loss) income per share		
Basic	68,142,158	67,234,465
Diluted	68,142,158	69,991,340

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

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

	Year Ended December 31,	
	2024	2023
Net (loss) income	$ (301,743)	$ 49,271
Other comprehensive (loss) income:		
Foreign currency translation (loss) gain	(396)	188
Net unrealized gain on marketable securities	107	95
Other comprehensive (loss) income	(289)	283
Comprehensive (loss) income	$ (302,032)	$ 49,554

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

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

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of January 1, 2023	66,512,011	$ 7	$ 694,906	$ (86)	$ (354,220)	$ 340,607
Issuance of common stock upon exercise of stock options	1,010,376	—	5,725	—	—	5,725
Issuance of common stock upon vesting of RSUs	185,045	—	—	—	—	—
Stock-based compensation expense	—	—	37,546	—	—	37,546
Other comprehensive income	—	—	—	283	—	283
Net income	—	—	—	—	49,271	49,271
Balance as of December 31, 2023	67,707,432	7	738,177	197	(304,949)	433,432
Issuance of common stock upon exercise of stock options	210,088	—	327	—	—	327
Issuance of common stock upon vesting of RSUs	712,218	—	—	—	—	—
Stock-based compensation expense	—	—	33,038	—	—	33,038
Other comprehensive loss	—	—	—	(289)	—	(289)
Net loss	—	—	—	—	(301,743)	(301,743)
Balance as of December 31, 2024	68,629,738	$ 7	$ 771,542	$ (92)	$ (606,692)	$ 164,765

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

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

	Year Ended December 31,	
	2024	**2023**
Cash flows (used in) provided by operating activities:		
Net (loss) income	$ (301,743)	$ 49,271
Adjustments to reconcile net (loss) income to net cash (used in) provided by operating activities:		
Stock-based compensation expense	33,038	37,161
Depreciation expense	904	1,088
Accretion of investment discounts, net	(9,856)	(9,940)
Inventory impairment and loss on firm purchase commitments	118,680	—
Property and equipment impairment	958	—
Charge for purchase of IPR&D assets	36,203	—
Changes in operating assets and liabilities:		
Accounts receivable, net	39,602	(24,744)
Inventories	(9,253)	(73,129)
Prepaid expenses and other assets	4,988	(3,226)
Accounts payable	(19,102)	15,882
Accrued expenses	(59,810)	21,597
Operating lease liabilities	(2,256)	(2,041)
Net cash (used in) provided by operating activities	(167,647)	11,919
Cash flows provided by investing activities:		
Purchases of property and equipment	(157)	(1,241)
Purchases of IPR&D assets, including transaction costs	(36,203)	—
Purchases of investments	(231,986)	(300,826)
Proceeds from maturities of marketable securities	344,000	394,120
Net cash provided by investing activities	75,654	92,053
Cash flows provided by financing activities:		
Follow-on offering costs paid	—	(136)
Proceeds from exercise of stock options and RSUs vesting	2,132	6,994
Withholding taxes paid on stock-based awards	(1,784)	(3,315)
Net cash provided by financing activities	348	3,543
Effect of exchange rate changes on cash, cash equivalents and restricted cash equivalents	(438)	160
Net (decrease) increase in cash, cash equivalents and restricted cash equivalents	(92,083)	107,675
Cash, cash equivalents and restricted cash equivalents, beginning of year	170,920	63,245
Cash, cash equivalents and restricted cash equivalents, end of year	$ 78,837	$ 170,920
Reconciliation of cash, cash equivalents and restricted cash equivalents:		
Cash and cash equivalents	$ 77,391	$ 170,201
Restricted cash equivalents	1,446	719
Total cash, cash equivalents and restricted cash equivalents:	$ 78,837	$ 170,920
Supplemental disclosure of cash flow information:		
Taxes withheld on stock-based awards included in accrued expenses	$ 2	$ 23
Purchases of property and equipment included in accounts payable	$ —	$ 20
Income taxes paid	$ 708	$ 6,389

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

AMYLYX PHARMACEUTICALS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. NATURE OF BUSINESS

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

Risks and Uncertainties

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

The accompanying consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. The Company expects to continue to generate operating losses for the foreseeable future. In January 2025, the Company received gross proceeds of $69 million in a public offering of the Company's common stock (see Note 18 *Subsequent events*). The Company expects that its cash, cash equivalents, marketable securities and the proceeds from the January 2025 Offering will be sufficient to fund its operating expenses and capital expenditure requirements for at least 12 months from the issuance of these consolidated financial statements.

To continue its development efforts, the Company will need to obtain substantial additional funding through public or private equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements in order to fund its research and development and ongoing operating expenses. The Company may not be able to obtain financing on acceptable terms, when needed or at all, and the Company may not be able to enter into collaborations, strategic alliances or licensing arrangements. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. Any collaborations, strategic alliances or licensing arrangements may require the Company to relinquish rights to certain of its technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to the Company. If the Company is unable to obtain funding, the Company could be forced to delay, limit, reduce or eliminate some or all of its research and development programs, pipeline expansion or future commercialization efforts or grant rights to develop and market product candidates, which could adversely affect its business prospects. Although management will continue to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations when needed or at all.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation and Consolidation—The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the U.S., or GAAP, and include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification, or ASC, and Accounting Standards Updates, or ASU, of the Financial Accounting Standards Board, or FASB.

Use of Estimates—The preparation of the 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.

Revenue recognition—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 in July 2022. In September 2022, AMX0035 received approval as RELYVRIO by the FDA for the treatment of ALS in adults, and the Company launched RELYVRIO in the U.S. in October 2022. In April 2024, the Company announced that it had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO and ALBRIOZA (AMX0035) for ALS and remove the product from the market based on topline results from the global Phase 3 PHOENIX trial, which did not meet its prespecified primary and secondary endpoints. Amylyx wound down the Open Label Extension as planned.

The Company entered into arrangements with wholesalers, specialty pharmacies and specialty distributors, or customers, to distribute ALBRIOZA, RELYVRIO, prior to voluntary discontinuation. In accordance with ASC Topic 606 - *Revenue from Contracts with Customers*, or Topic 606, revenue is recognized when the customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to be entitled to in exchange for those goods or services.

At contract inception, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Product Revenue, Net

The Company sold its approved products to its customers. These customers subsequently resold our products to specialty pharmacy providers, specialty distributors, health care providers, certain medical centers or hospitals, and patients. In addition to agreements with the customers, the Company entered into arrangements with specialty pharmacies, health care providers and payors that provide for government mandated and/or privately negotiated rebates with respect to the purchase of our products. The Company's customer identification process considered a number of factors, including contractual and legal factors, and who controls the Company's product and bears inventory risk. The Company evaluated these factors on a customer-by-customer basis to determine the appropriate customer for revenue recognition purposes. In some cases, the Company used a third-party logistics providers to deliver the Company's product to its customers, but the Company recognized revenue upon delivery to the customer, as it was determined that the third-party logistics provider was acting as our agent.

The Company recognizes revenue on product sales when the customer obtains control of our product, which occurs at a point in time (upon delivery). Product revenues are recorded net of applicable GTN adjustments, which are described below.

Taxes collected from customers relating to product sales and remitted to governmental authorities are excluded from revenue. The Company expenses incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that the Company would have recognized is one year or less. However, no such costs were incurred during the years ended December 31, 2024 and 2023.

GTN Adjustments

Revenues from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration related to certain GTN adjustments. Components of GTN adjustments include trade discounts and allowances, product returns, third-party payor rebates, and other allowances that are offered within contracts between the Company, its customers and payors relating to the sale of our products. These GTN adjustments, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a Customer). These estimates take into consideration a range of possible outcomes which are probability-weighted in accordance with the expected value method in Topic 606 for relevant factors such as historical experience, payer channel mix (e.g., Medicare or

Medicaid), current contract prices under applicable programs, unbilled claims and processing time lags and inventory levels in the distribution channel. In certain circumstances, the Company applies the most likely method in Topic 606. The determination to use the expected value method or the most likely method is based on the type of GTN adjustment and what method better predicts the amount of consideration we expect to be entitled to. Overall, these GTN adjustments reflect in the transaction price the amount of consideration to which the Company expects to be entitled to in exchange for transferring promised goods or services to its customers.

The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under the contract will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, the Company will adjust these estimates, which would affect product revenue, net and earnings in the period such variances become known.

Trade Discounts and Allowances

The Company generally provides customers with prompt payment discounts and pay fees for distribution services and for certain data that distributors provide to us that are explicitly stated in our contracts and are recorded as a reduction of revenue in the period the related product revenue is recognized. Payment from customers is typically due within 30 calendar days of the invoice date, without consideration to the prompt payment discounts.

Product Returns

Consistent with industry practice, the Company generally offers customers a limited right of return for product that has been purchased from the Company based on the product's expiration date, which is set to lapse within a specified period stated in the contract. Additionally, our limited right of return policy allows for eligible returns from customers in circumstances where product was shipped in error or was damaged in shipping, or product was returned pursuant to an official drug recall.

The Company estimates the amount of product sales that may be returned by our customers and records this estimate as a reduction of revenue in the period the related product revenue is recognized, as well as reductions to accounts receivable, net on the consolidated balance sheets.

Provider Chargebacks and Discounts

Chargebacks for fees and discounts to providers represent the estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to customers who directly purchase the product from the Company. Customers charge the Company for the difference between what they pay for the product and the ultimate selling price to the qualified healthcare providers. These GTN adjustments are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable, net. GTN adjustments for chargebacks consist of credits that customers have not claimed, but for which we expect to issue a credit.

Payor Rebates

The Company contracts with certain government and private payor organizations, primarily government and commercial health insurance companies, for the payment of rebates with respect to utilization of our products. The Company is subject to discount obligations under state Medicaid programs and Medicare. These GTN adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses and other current liabilities on the consolidated balance sheets. For Medicare, the Company also estimates the number of patients in the prescription drug coverage gap for whom it will owe an additional liability under the Medicare Part D program. The Company's liability for these rebates consists of invoices received for claims from prior quarters that have not been paid or for which an invoice has not yet been received, estimates of claims for the current quarter, and estimated future claims that will be made for product that has been recognized as revenue, but which remains in the distribution channel inventories at the end of each reporting period.

Other Incentives

Other incentives which the Company offers include voluntary patient assistance programs, such as its co-pay assistance program, which are intended to provide financial assistance to qualified commercially-insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that the Company expects to receive associated with product that has been recognized as revenue for each reporting period. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accrued expenses and other current liabilities on the consolidated balance sheets.

Comprehensive Income (Loss)—Comprehensive income (loss) includes net loss, as well as other changes in stockholders' equity that result from transactions and economic events other than those with stockholders. Comprehensive income (loss) is composed of net income (loss) and other comprehensive (loss) income. Other comprehensive (loss) income consists of unrealized gains and losses on marketable securities and foreign currency translation.

Cash and Cash Equivalents—The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. Cash equivalents represent funds invested in readily available checking and money market funds.

Restricted Cash Equivalents— Restricted cash equivalents consist of cash serving as collateral for a letter of credit issued for the Company's office spaces and collateral for a corporate credit card program.

Accounts receivable, net— The Company's accounts receivable consists of amounts due from customers related to product sales and have standard payment terms. The Company analyzes accounts that are past due for collectability and provides reserves against accounts receivable for expected credit losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. The credit losses were not material in the periods presented.

Marketable Securities—Marketable securities are composed of U.S. treasury bills and U.S. agency bonds. The Company classifies all of its marketable securities as available-for-sale. Accordingly, these investments are recorded at fair value, which is determined based on quoted market prices. Unrealized gains and losses on available-for-sale securities are included as a separate component of other accumulated comprehensive loss. The cost of marketable securities is adjusted for amortization of premiums and accretion of discounts. Such amortization and accretion are included in interest income. Realized gains and losses are included in other expense, net. The Company evaluates marketable securities for other-than-temporary impairment at the balance sheet date. Declines in fair value, if any, determined to be other than temporary-than-temporary are also included in other income, net.

When assessing marketable securities for other-than-temporary declines in value, the Company considers such factors as, among other things, how significant the decline in value is as a percentage of the original cost, how long the market value of the investment has been less than its original cost, and the Company's ability and intent to retain the investment for a period of time sufficient to allow for any anticipated recovery in fair value and market conditions in general. There were no impairment charges on marketable securities in the periods presented.

Concentrations of Credit Risk—Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents, marketable securities and accounts receivable, net. The Company maintains its cash in financial institutions that management believes have high credit quality. The Company has not experienced any losses on such accounts, and does not believe it is exposed to any unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company's accounts receivable, net represents amounts due to the Company from customers. Amylyx performs ongoing credit evaluations of its customers and generally does not require collateral. The Company monitors its exposure and records a reserve against uncollectible amounts as necessary. Five and three customers individually accounted for approximately 89% and 81% in the aggregate of gross product revenue in 2024 and 2023, respectively. One and three customers individually accounted for approximately 100% and 81% of total accounts receivable, net as of December 31, 2024 and 2023, respectively.

Fair Value Measurements—Assets and liabilities recorded at fair value on a recurring basis on the consolidated balance sheet are categorized based upon the level of judgment associated with the inputs used to measure fair values. Fair

value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

- ***Level 1***—Quoted prices in active markets for identical assets or liabilities.
- ***Level 2***—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- ***Level 3***—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The Company's financial instruments consist of cash, cash equivalents, restricted cash, marketable securities, accounts receivable, net, accounts payable and accrued expenses. The Company's marketable securities are carried at fair value, determined according to Level 1 and Level 2 inputs to the fair value hierarchy described above. The remaining financial instruments are stated at their respective carrying amounts, which approximate fair value due to the short-term nature of these assets and liabilities.

Inventories—The Company values its inventories at the lower of cost or estimated net realizable value. The Company determines the cost of its inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. The Company classifies inventory as long-term when consumption or sale of the inventory is expected beyond its normal operating cycle of twelve months. The Company performs an assessment of the recoverability of capitalized inventory during each reporting period, and it writes down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges are recorded within cost of sales.

The Company capitalizes inventory costs associated with the Company's products after regulatory approval when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized. Inventory acquired prior to receipt of regulatory approval of a product candidate is expensed as research and development expense as incurred. Inventory that can be used in either the production of clinical or commercial product is initially capitalized and subsequently expensed as research and development expense when identified for use in the manufacture of drugs still in development.

Property and Equipment, net—Property and equipment are stated at cost, net of accumulated depreciation. Depreciation of property and equipment is calculated using the straight-line method over the estimated useful lives of the respective assets. Maintenance and repairs that do not improve or extend the life of the assets are expensed when incurred. Upon sale or retirement of assets, the cost and accumulated depreciation are removed from the consolidated balance sheets and any resulting gain or loss is reflected in the consolidated statements of operations in the period realized. The range of useful lives of property and equipment is as follows:

	Estimated Useful Life
Leasehold improvements	Lesser of the estimated life or remaining lease term
Furniture and fixtures	4 years
Computer hardware and software	3 years
Construction in progress	Not depreciated

Impairment of Long-Lived Assets—The Company evaluates assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to

generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book values of the assets exceed fair value.

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

Research and Development—Research and development expenses include costs directly attributable to the conduct of research and development activities. Expenditures relating to research and development are expensed in the period incurred. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made. In addition, research and development-related salaries and benefits, facility, and overhead costs, supplies and other related costs are included in research and development expense.

License and Collaboration Agreements —The Company analyzes license and collaboration arrangements pursuant to ASC Topic 808 - *Collaborative Arrangement Guidance and Considerations*, or ASC 808, to assess whether such arrangements, or transactions between arrangement participants, involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities.

Collaboration arrangements often require upfront payments and may include additional milestone, research and development cost sharing, royalty or profit share payments, contingent upon the occurrence of certain future events linked to the success of the asset in development and commercialization. Upfront payments associated with collaborative arrangements and subsequent payments made to the partner for the achievement of development milestones prior to regulatory approval are expensed to acquired IPR&D expense as incurred. Contingent consideration obligations are recorded when it is probable that they will occur and they can be reasonably estimated.

Sales and Marketing Costs—Sales and marketing expenses consist primarily of wages and benefits for sales and marketing personnel, professional and consulting fees, administrative travel expenses, and marketing and advertising costs such as marketing literature, promotional activities, conferences and seminars and branding. Sales and marketing, and advertising costs are expensed as incurred and included in selling, general and administrative expenses in the accompanying consolidated statements of operations. The Company considers advertising costs as expenses related to the promotion of the Company's commercial products. For the years ended December 31, 2024 and 2023, advertising costs were $2.7 million and $9.5 million, respectively.

Patent-Related Costs—Patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as selling, general and administrative expenses in the accompanying consolidated statements of operations.

Stock-Based Compensation Expense—Stock-based compensation is recognized in the consolidated statements of operations based on the fair value on the date of grant over the requisite service period, which is generally equal to the vesting period of the respective award. Forfeitures are accounted for as incurred. Generally, the Company issues stock awards with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company classifies stock-based compensation expense in the same manner in which the award recipient’s payroll costs are classified.

The fair value of each restricted common stock award is measured based on the fair value of the Company's common stock on the grant date.

The fair value of each option grant is estimated on the date of grant using the Black-Scholes option pricing model, which requires inputs based on certain subjective assumptions, including the expected stock price volatility, the expected term of the award, the risk-free interest rate, and expected dividends. The Company estimates its expected stock price volatility based on the historical volatility of publicly traded peer companies. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. There is no expected dividend yield since the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future. The stock price of the Company is based on the closing price on the date of grant.

Contingencies—From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of business activities. The Company accrues for loss contingencies when losses become probable and are reasonably estimable. If the reasonable estimate of the loss is a range and no amount within the range is a better estimate, the minimum amount of the range is recorded as a liability on the Company's consolidated balance sheets. The Company does not accrue for contingent losses that, in its judgment, are considered to be reasonably possible, but not probable; however, it discloses the range of reasonably possible losses.

Leases—The Company leases its offices, and may from time to time, enter into other lease agreements in conducting its business. The Company determines if an arrangement includes a lease at the inception of the agreement. For each of the Company's lease arrangements, the Company records a right-of-use asset representing the Company's right to use an underlying asset for the lease term and a lease liability representing the Company's obligation to make lease payments. Operating lease right-of-use assets and operating lease liabilities are recognized at the lease commencement date based on the net present value of the remaining future minimum lease payments over the lease term. If the interest rate implicit in the Company's leases is not readily determinable, in determining the weighted-average discount rate used to calculate the net present value of lease payments, the Company utilizes an estimate of its incremental borrowing rate based on market sources including interest rates for companies with similar credit quality for agreements of similar duration to discount the lease payments. Lease expense for the Company's operating leases is recognized on a straight-line basis over the lease term and variable lease costs are expensed as incurred. The Company did not have financing leases as of December 31, 2024 and 2023.

The Company elected the practical expedient not to apply the recognition and measurement requirements to short-term leases, which is any lease with a term of one year or less as of the lease commencement date. The lease may require the Company to pay additional amounts for maintenance and other expenses, which are generally referred to as non-lease components. 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. If a lease includes options to extend the lease term, the Company does not assume the option will be exercised in its initial lease term assessment unless there is reasonable certainty that the Company will renew based on an assessment of economic factors present as of the lease commencement date.

Income Taxes—The Company accounts for income taxes using the asset and liability approach. Deferred tax assets and liabilities represent future tax consequences of temporary differences between the financial statement carrying amounts and the tax basis of assets and liabilities and for loss carryforwards using enacted tax rates expected to be in effect in the years in which the differences reverse. A valuation allowance is established to reduce deferred tax assets to the amounts expected to be realized. The Company also recognizes a tax benefit from uncertain tax positions only if it is "more likely than not" that the position is sustainable based on its technical merits. The Company accounts for interest and penalties related to uncertain tax positions as part of its provision for income taxes. To date, the Company has not incurred material interest and penalties related to income tax positions.

Valuation allowances are provided, if, based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. As of December 31, 2024, we continued to maintain a full valuation allowance against all of our deferred tax assets based on management's evaluation of all available evidence, including our history of incurring significant losses from operations.

Recent Accounting Pronouncements

New Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvement to Income Tax Disclosures*, or ASU 2023-09, to enhance the transparency and decision usefulness of income tax disclosures. ASU 2023-09 is effective for the Company beginning January 1, 2025 on a prospective basis. The impact of this ASU on the tax disclosures is not expected to be material.

In November 2024, the FASB issued ASU No. 2024-03, Income Statement – Reporting Comprehensive Income – Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses, which requires new financial statement disclosures in tabular format, in the notes to financial statements, of specified information about certain costs and expenses. The amendments in this update do not change or remove current expense disclosure requirements. The amendments in this update are effective for the Company's annual financial statement disclosure beginning December 31, 2027, and interim periods within the years beginning January 1, 2028. Early adoption is permitted. The Company is currently evaluating the impact of this ASU on its consolidated financial statements and related disclosures.

3. PRODUCT REVENUE, NET

To date, the Company's only source of product revenue had been from the sales of RELYVRIO, known as ALBRIOZA in Canada. In April 2024, the Company announced it had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ and remove the product from the market in the U.S. and Canada based on topline results from the Phase 3 PHOENIX trial. The following table reconciles gross product revenue to net product revenue:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Product revenue, gross	$ 114,265	$ 431,433
GTN adjustments	(26,894)	(50,647)
Product revenue, net	$ 87,371	$ 380,786

The activity and ending reserve balance for GTN adjustments were as follows for the periods indicated:

	Chargebacks and Cash Discounts	Medicaid and Medicare Rebates	Other Rebates, Returns, Discounts and Adjustments	Total
		(in thousands)		
Balance at January 1, 2022	$ 648	$ 1,992	$ 1,664	$ 4,304
Provision related to sales in the current year	17,898	10,887	22,378	51,163
Adjustments related to prior period sales	(280)	(236)	—	(516)
Credits and payments made	(15,123)	(7,697)	(12,969)	(35,789)
Balance at December 31, 2023	$ 3,143	$ 4,946	$ 11,073	$ 19,162
Provision related to sales in the current year	4,983	3,402	21,333	29,718
Adjustments related to prior period sales	(1,696)	(191)	(961)	(2,848)
Credits and payments made	(6,430)	(8,157)	(25,665)	(40,252)
Balance at December 31, 2024	$ —	$ —	$ 5,780	$ 5,780

Included in the ending reserve balance for GTN adjustments are chargebacks resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to customers who directly purchase the product from the Company, rebates and estimates for product returns. Chargebacks, discounts and returns are recorded as reductions of accounts receivable, net on the condensed consolidated balance sheets to the extent there are receivable balances to reduce. If there are net balances owed to customers, they are recorded as a component of accrued expenses on the consolidated balance sheets.

4. MARKETABLE SECURITIES

The Company has classified all of its marketable securities as "available-for-sale". The Company records available-for-sale securities at fair value, with the unrealized gains and losses included as a separate component of other accumulated comprehensive income (loss). There were no realized gains or losses recognized in the periods presented.

The Company adjusts the cost of available-for-sale debt securities for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion are included in interest income. The cost of securities sold is based on the specific identification method. The Company includes interest and dividends on securities classified as available-for-sale in interest income. Accrued interest receivable relating to the Company's available-for-sale securities is presented within prepaid expenses and other current assets in the accompanying consolidated balance sheets, and amounted to $0.1 million and $0.5 million at December 31, 2024 and 2023, respectively.

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

December 31, 2024	Amortized Cost Basis	Unrealized Gain	Unrealized Loss	Fair Values
		(in thousands)		
Treasury bills	$ 98,939	$ 171	$ —	$ 99,110
Total marketable securities	$ 98,939	$ 171	$ —	$ 99,110

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

5. INVENTORIES

Inventories consisted of the following:

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

In April 2024, the Company announced it had started a process with the FDA and Health Canada to voluntarily discontinue the marketing authorizations for RELYVRIO®/ALBRIOZA™ and remove the product from the market in the U.S. and Canada based on topline results from the global Phase 3 PHOENIX trial. As a result, the Company recorded approximately $92.5 million of charges associated with the write-down of inventory for the year ended December 31, 2024. For the year ended December 31, 2023, the Company recognized inventory write-downs of $3.3 million. Inventory amounts written down as a result of obsolescence or other reasons are charged to cost of sales.

6. PROPERTY AND EQUIPMENT, NET

Property and equipment, net consisted of the following:

	December 31,	
	2024	2023
	(in thousands)	
Furniture and fixtures	$ 382	$ 382
Computer hardware and software	1,541	3,167
Leasehold improvements	176	176
Construction in progress	221	589
Total property and equipment	2,320	4,314
Less: accumulated depreciation	(1,359)	(1,628)
Total property and equipment, net	$ 961	$ 2,686

7. ACCRUED EXPENSES

Accrued expenses consisted of the following:

	December 31,	
	2024	2023
	(in thousands)	
Accrued external research and development	$ 4,353	$ 12,625
Accrued benefits and incentive compensation	9,992	16,790
Accrued manufacturing	500	1,652
Accrued consulting and other professional fees	1,974	6,506
Accrued returns, rebates and co-pay assistance	5,334	16,063
Accrued royalties	—	3,111
Accrued loss on future purchase commitments	1,538	—
Other accrued expenses	258	977
Total accrued expenses	$ 23,949	$ 57,724

8. FAIR VALUE MEASUREMENTS

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

	December 31, 2024			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
Assets:				
Cash equivalents	$ 37,550	$ —	$ —	$ 37,550
Restricted cash equivalents	1,446	—	—	1,446
Treasury bills	99,110	—	—	99,110
Total financial assets	$ 138,106	$ —	$ —	$ 138,106

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

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

9. LEASES

The Company leases its office facilities under non-cancelable operating leases that expire at various dates through October 2026.

On September 12, 2024, the Company entered into a new office lease in Cambridge, Massachusetts for office space for its headquarters facility. The lease commencement date is expected to be June 1, 2025 when the premises are expected to be available for occupancy and, therefore, as the office lease has not commenced, the related operating lease right-of-use assets and liabilities are not recorded in the Company's consolidated balance sheet as of December 31, 2024.

Components of lease expense required by ASC 842 are presented below for the years ended December 31, 2024 and 2023:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Lease cost		
Operating lease cost	$ 2,175	$ 2,175
Total lease cost	$ 2,175	$ 2,175

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, the Company uses 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 the Company 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 the Company has 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 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. Variable lease payments during the years ended December 31, 2024 and 2023 were not material.

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

	December 31,	
	2024	2023
	(in thousands)	
Assets		
Operating lease right-of-use assets	$ 1,771	$ 3,725
Liabilities		
Operating lease right-of-use liabilities, current	$ 1,518	$ 2,257
Operating lease right-of-use liabilities, net of current portion	463	1,980
Total operating lease liabilities	$ 1,981	$ 4,237

During the years ended December 31, 2024 and 2023, the Company made cash payments for operating leases of $2.5 million and $2.4 million, respectively. Future minimum lease payments under non-cancelable leases as of December 31, 2024, were as detailed below (in thousands):

	As of December 31, 2024
2025	$ 1,587
2026	476
2027	—
2028	—
2029	—
Total undiscounted lease payments	2,063
Less: imputed interest	(82)
Total operating lease liabilities	$ 1,981

As of December 31, 2024 and 2023, the weighted average remaining lease term was 1.2 years and 2 years, respectively. As of December 31, 2024 and 2023, the weighted average incremental borrowing rate used to determine the operating lease right-of-use assets was 7.4% and 7.3%, respectively.

10. ASSET ACQUISITIONS AND COLLABORATION AGREEMENTS

Eiger Asset Acquisition

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

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

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

As part of the transaction, the Company assumed certain contractual obligations from Eiger, including royalty obligations between 4% and 7% on future sales owed to certain academic institutions and individuals. The Company will recognize these royalty payments related to avexitide in the period in which the achievement of the underlying milestones becomes probable.

Gubra A/S Collaboration and License Agreement

On December 23, 2024, the Company entered into a collaboration and license agreement, or the Gubra Agreement, with Gubra pursuant to which the parties will perform research and discovery activities for the development of a potential novel long-acting GLP-1 receptor antagonist, under the oversight of a joint research committee. The collaboration provides the Company an exclusive license to develop, manufacture, commercialize and otherwise exploit any development candidate and product(s) arising in the performance of activities under the agreement.

The Company made an immaterial upfront payment in January 2025, which became due upon the effective date of the Gubra Agreement. Since the payment was made for the use of Gubra's intellectual property and research and development services and there is no alternative use, the Company recorded the upfront payment to research and development expense on the consolidated statements of operations in 2024. Gubra is eligible to receive an additional $53.5 million upon the achievement of certain development, regulatory and commercial milestones, as well as tiered royalties on future sales from any products that result from the agreement. None of these payments are deemed probable at December 31, 2024 and have not been recognized. The Company has agreed to make quarterly payments to fund Gubra's ongoing research activities, which are not expected to be material.

11. STOCK OPTION AND GRANT PLANS

Stock Incentive Plan—In January 2022, the Company's board of directors adopted, and its stockholders approved the 2022 Stock Option and Incentive Plan, or 2022 Plan. Under the 2022 Plan, the Company may grant incentive stock options, or ISOs, non-statutory stock options, stock appreciation rights, restricted stock units, restricted stock awards and other stock-based awards. As of December 31, 2024, there were 7,080,463 shares available for future issuance under the 2022 Plan. The options issued under the 2022 Plan expire 10 years following the date of grant. Stock options and restricted stock units typically vest over 4 years. We recognize the compensation cost of awards subject to service-based vesting conditions over the requisite service period, which is generally equal to the vesting period of the respective award.

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.

Inducement Plan—In July 2023, the Company's board of directors adopted the Amylyx Pharmaceuticals, Inc. 2023 Inducement Plan, or the Inducement Plan, to grant equity awards to induce highly-qualified prospective officers and employees who are not currently employed by the Company to accept employment and provide them with a proprietary interest in the Company. The Company has reserved 750,000 shares of its common stock that may be issued under the Inducement Plan. As of December 31, 2024, there were 360,167 shares available for future issuance under the Inducement Plan.

Employee Stock Purchase Plan—In January 2022, the Company's board of directors adopted the 2022 Employee Stock Purchase Plan, or ESPP, which was subsequently approved by the Company's stockholders. The ESPP initially reserves and authorizes the issuance of up to a total of 605,000 shares of common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically increase each January 1, beginning on January 1, 2023 and each January 1 thereafter through January 1, 2032, by the least of (i) 1% of the outstanding number of shares of our common stock on the immediately preceding December 31, (ii) 1,210,000 shares or (iii) such number of shares of common stock as determined by the ESPP administrator. The initial purchase period under the ESPP has not yet commenced. As of December 31, 2024, there were 1,947,194 shares available for future issuance under the ESPP.

The Company estimates the fair value of stock option awards on the grant date using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year Ended December 31,	
	2024	2023
Risk-free interest rate	4.44%	3.77%
Expected term (in years)	6.08	6.05
Expected volatility	69.17%	70.35%
Dividend yield	0.00%	0.00%

The weighted average grant date fair value of stock options granted during the year ended December 31, 2024 and 2023 was $5.61 per share and $19.56 per share, respectively.

A summary of option activity for the year ended December 31, 2024, is as follows:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at January 1, 2024	9,823,248	$ 18.39	7.9	$ 27,639
Granted	3,072,661	$ 8.54		
Exercised	(210,088)	$ 1.55		
Cancelled or forfeited	(4,957,114)	$ 19.30		
Outstanding at December 31, 2024	7,728,707	$ 14.34	7.5	$ 3,584
Exercisable at December 31, 2024	3,671,292	$ 15.79	6.2	$ 587
Unvested at December 31, 2024	4,057,415	$ 13.03	8.7	$ 2,998

The aggregate intrinsic value of options exercised during the years ended December 31, 2024 and 2023 was $1.0 million and $20.6 million, respectively.

The total fair value of stock options vested during the years ended December 31, 2024 and 2023 was $32.5 million and $31.2 million, respectively.

Restricted Stock Unit Activity

A summary of restricted stock unit activity for the year ended December 31, 2024, is as follows:

	Number of shares	Weighted Average Grant Date Fair Value
Nonvested as of January 1, 2024	1,112,542	$ 24.80
Granted	2,865,128	$ 8.74
Vested	(712,218)	$ 10.88
Forfeited	(1,052,547)	$ 19.61
Nonvested as of December 31, 2024	2,212,905	$ 10.96

Stock-Based Compensation Expense—The Company recorded stock-based compensation expense in the following expense categories of its statements of operations:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Research and development expenses	$ 8,758	$ 9,843
Selling, general and administrative expenses	24,280	27,318
Total stock-based compensation	$ 33,038	$ 37,161

The following table summarizes unrecognized stock-based compensation expense as of December 31, 2024, 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 December 31, 2024	
	Unrecognized Expense (in thousands)	Weighted-average Recognition Period (in years)
Stock options	$ 31,627	2.14
Restricted stock units	$ 17,892	2.50

12. INCOME TAXES

The components of net (loss) income before the provision for income taxes are as follows:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
U.S.	$ (301,757)	$ 52,263
Non-U.S.	(379)	2,034
(Loss) income before income taxes	$ (302,136)	$ 54,297

The (benefit) provision for income taxes is as follows:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Current income tax provision		
U.S. - Federal	$ 130	$ 1,219
U.S. - State	23	2,839
Non-U.S.	(770)	1,192
	$ (617)	$ 5,250
Deferred income tax provision		
Non-U.S.	$ 224	$ (224)
Provision for income taxes	$ (393)	$ 5,026

A reconciliation of the Company's effective income tax rate to the U.S. statutory federal income tax rate of 21% for the years ended December 31, 2024 and 2023 is as follows:

	Year Ended December 31,	
	2024	2023
Tax at U.S. statutory tax rate	21.0%	21.0%
State income tax benefit	4.0%	3.3%
Research and development tax credits	2.7%	(12.6)%
Executive Compensation	(0.8)%	6.2%
Uncertain Tax Positions	(0.5)%	2.1%
Valuation allowances	(24.7)%	(12.2)%
Stock Based Compensation	(1.2)%	(0.5)%
Other	(0.4)%	2.0%
Effective income tax rate	0.1%	9.3%

Deferred tax assets and liabilities were as follows:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Deferred tax assets:		
Federal net operating loss carryforwards	$ 55,174	$ 14,667
State net operating loss carryforwards	7,881	8,164
Capitalized research and development costs	61,280	39,297
Inventory	—	1,090
Tax credits	16,005	8,039
Stock Based Compensation	3,396	3,792
Intangibles	7,507	—
Accruals and other	7,906	10,425
Total deferred tax assets	$ 159,149	$ 85,474
Valuation allowance	(158,542)	(83,922)
Net total deferred tax assets	$ 607	$ 1,552
Deferred tax liabilities:		
Other	(607)	(1,328)
Total deferred tax liabilities	$ (607)	$ (1,328)
Net deferred tax assets	$ —	$ 224

On a periodic basis the Company reassess the valuation allowance that has been established, weighing all positive and negative evidence. As of December 31, 2024, the Company maintained a full valuation against net deferred tax assets.

As of December 31, 2024 and 2023, the Company had federal NOL loss carryforwards of approximately $262.7 million and $69.8 million, respectively, and state NOL loss carryforwards of approximately $126.9 million and $124.6 million, respectively, which are available to reduce future taxable income. All U.S. federal NOL carryforwards as of December 31, 2024 carry forward indefinitely. Of the $126.9 million state NOL carryforwards, $119.9 million relate to Massachusetts and begin to expire in 2040. As of December 31, 2024 and 2023, the Company also had federal tax credits of $13.4 million and $6.8 million, respectively, and state tax credits of $3.3 million and $1.6 million, respectively. The tax credit carryforwards will expire at various dates beginning in 2035.

The utilization of NOL and research and development tax credit carryforwards may be subject to a substantial annual limitation under Sections 382 and 383 of the IRC. Ownership changes occurred in the years ended December 31, 2016 and 2023. These ownership changes do not impact the Company's overall ability to utilize NOL carryforwards and research and development tax credit carryforwards but may limit the amount that can be utilized annually to offset future taxable income.

The following table reflects the roll-forward of the Company's valuation allowance for the years ended December 31, 2024 and 2023:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Valuation allowance at beginning of year	$ 83,922	$ 90,587
Increases (decreases) recorded to income tax provision	74,620	(6,665)
Valuation allowance at end of year	$ 158,542	$ 83,922

The increase in the valuation allowance recorded during the year was primarily due to the increase in net operating loss generated by the Company in 2024 and required capitalization of research and development costs.

The Company accounts for uncertainty in income taxes under the provisions of ASC 740 which defines the thresholds for recognizing the benefits of tax return positions in the consolidated financial statements as "more likely than not" to be sustained by the taxing authority. The tax benefit is measured based on the largest benefit that has a greater than 50%

likelihood of being realized upon ultimate settlement. A reconciliation of the beginning and ending amount of gross unrecognized tax benefits is as follows:

	Year Ended December 31,	
	2024	2023
	(in thousands)	
Balance at beginning of the period	$ 2,209	$ 1,013
Increases related to tax positions taken during prior years	55	271
Increases related to tax positions taken during the current year	1,498	925
Balance at end of the period	$ 3,762	$ 2,209

The Company has reviewed the tax positions taken, or to be taken, in its tax returns for all tax years currently open to examination by a taxing authority. All uncertain tax benefits, if recognized, would impact the effective tax rate if recognized, offset by changes to the Company's valuation allowance which also would impact the effective tax rate. The Company does not expect the amount of unrecognized tax benefits to materially change over next 12 months. The Company accrues interest and penalties related to unrecognized tax benefits as a component of its provision for income taxes. The Company did not recognize any material interest or penalties related to uncertain tax positions during the years ended December 31, 2024 and 2023.

The Company files U.S. federal, foreign and state income tax returns in various jurisdictions. The status of limitations varies by jurisdiction. There are currently no federal or state audits or examinations in process.

13. EMPLOYEE BENEFIT PLANS

The Company maintains a tax-qualified retirement plan that provides eligible U.S. employees with an opportunity to save for retirement on a tax-advantaged basis. Plan participants are able to defer eligible compensation subject to applicable annual IRC limits. The Company made $2.8 million and $2.3 million of contributions for the years ended December 31, 2024 and 2023, respectively.

14. NET (LOSS) INCOME PER SHARE

Net (Loss) Income per Share

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

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

	December 31,	
	2024	2023
Numerator:		
Net (loss) income	$ (301,743)	$ 49,271
Denominator:		
Weighted-average shares used to compute basic net (loss) income per share	68,142,158	67,234,465
Dilutive effect of employee stock options and restricted stock units	—	2,756,875
Weighted-average shares used to compute diluted net (loss) income per share	68,142,158	69,991,340
Net (loss) income per share		
Basic	$ (4.43)	$ 0.73
Diluted	$ (4.43)	$ 0.70

All stock options and restricted stock units were excluded from the computation of diluted weighted-average shares outstanding because such securities would have an antidilutive impact for the year ended December 31, 2024. The following stock options and restricted stock units outstanding at each period end have been excluded from the calculation of diluted net (loss) income per share because their inclusion would have been antidilutive:

	December 31,	
	2024	2023
Options to purchase common stock	7,728,707	5,775,303
Restricted stock units	2,212,905	543,233
Total excluded common stock equivalents	9,941,612	6,318,536

15. SEGMENTS

The Company has one operating segment which is the business of researching and developing therapeutics for neurodegenerative diseases and endocrine conditions. Our operating segments are determined based on how our Co-Chief Executive Officers, Justin Klee and Joshua Cohen, who collectively serve as our chief operating decision makers ("CODM") manages our business, regularly accesses discrete financial information, and evaluates performance for operating decision-making purposes, including allocation of resources or capital to specific compounds or projects in line with the Company's overall strategies and goals. The Company's entire business is managed by a single management team, which reports to the CODM. The accounting policies of the Company's segment are the same as those described in Note 2 *Significant Accounting Policies*.

The CODM assesses segment performance and decides how to allocate resources based on consolidated net (loss) income that also is reported on the consolidated statement of operations. The CODM uses net income to monitor budget and forecast versus actual results in assessing segment performance and to evaluate income generated from segment assets in deciding how to allocate resources. The measure of segment assets is reported on the consolidated balance sheets as total consolidated assets. For the years ended December 31, 2024 and 2023, all of the Company's long-lived assets were held within the U.S.

The following table reconciles segment revenue and expenses to consolidated net loss (income) for the year ended December 31, 2024 and 2023 (in thousands):

	Year Ended December 31,	
	2024	2023
Product revenue, net	$ 87,371	$ 380,786
Less[1,5]:		
Cost of sales[2]	124,633	25,441
Direct research and development expenses by program:		
AMX0035 - ALS	36,727	60,843
AMX0035 - PSP	16,917	6,404
Avexitide	2,766	—
Other programs	8,698	13,870
Acquired in-process research and development	36,203	—
Personnel-related research and development[3]	38,976	47,070
Selling, general and administrative	114,331	188,356
Restructuring expenses	22,851	—
(Benefit) provision for income taxes	(393)	5,026
Interest income	(13,809)	(16,155)
Other segment items[4]	1,214	660
Net (loss) income	$ (301,743)	$ 49,271

[1.] The significant expense categories and amounts align with the segment-level information that is regularly provided to the CODM. As the Company has one reportable segment, there were no intersegment eliminations for the year ended December 31, 2024 and 2023.

[2.] Includes inventory impairment and loss on firm purchase commitments of $118.7 million and zero during year ended December 31, 2024 and 2023, respectively.

[3.] The Company does not allocate personnel and other similar costs to specific programs because these costs are deployed across multiple programs.

[4.] Other segment items primarily consists of net realized and unrealized losses on foreign exchange transactions

[5.] Depreciation and amortization expense of $0.9 million and $1.1 million during the years ended December 31, 2024 and 2023, respectively, are allocated across the significant expense captions.

16. COMMITMENTS AND CONTINGENCIES

Legal Proceedings—As of December 31, 2024, the Company is not a party to any legal proceedings that are expected to have a material impact on the Company's consolidated financial statements. 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 grant agreements with the ALS Association, ALS Finding a Cure Foundation, Alzheimer's Drug Discovery Foundation, Alzheimer's Association and Cure Alzheimer's Fund, or Grantors. 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. The Company does not expect to incur any costs under the agreements given the discontinuation of sales of RELYVRIO®/ALBRIOZA.

As disclosed in Note 10 *Asset Acquisitions and Collaboration Agreements,* the Company assumed royalty obligations from Eiger related to the acquisition of avexitide and Gubra A/S related to a collaboration arrangement. As the conditions that would trigger royalty payments under the agreements have not occurred, no amounts have been recorded in the consolidated financial statements.

Purchase Commitments—The Company enters into agreements in the normal course of business with contract manufacturing organizations for raw material purchases and manufacturing services. As of December 31, 2024, the amounts committed under these agreements are not material.

Following the discontinuation of the sales of RELYVRIO®/ALBRIOZA in 2024, the Company recognized a loss on purchase commitments of $26.1 million, which was recorded to cost of sales on the condensed consolidated statement of operations.

17. RESTRUCTURING

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

Restructuring expenses consists primarily of employee severance and termination benefits, contract termination costs, impairment of long-lived assets and other costs. Liabilities for costs associated with a restructuring activity are recognized when the liability is incurred and are measured at fair value. One-time employee severance and termination benefits are expensed at the date the entity notifies the employee of the plan. One-time termination benefits primarily include severance, continuation of health insurance coverage, and other benefits such as outplacement support services for a specified period of time.

In connection with the Restructuring Plan, the Company performed an impairment evaluation of its long-lived assets resulting in an impairment charge of $0.9 million during the year ended December 31, 2024 related to the impairment of capitalized internal-use software.

Restructuring expenses for the year ended December 31, 2024 included $21.8 million of severance and employee benefit costs and $1.0 million of other contract termination costs and impairment charges. All costs related to this restructuring activity were paid as of December 31, 2024, and the Company does not expect to incur costs in future periods for the Restructuring Plan.

18. SUBSEQUENT EVENTS

On January 13, 2025, the Company closed an underwritten public offering of 19,714,285 shares of its common stock at a public offering price of $3.50 per share. The net proceeds from this offering were approximately $65.5 million, after deducting underwriting discounts and commissions and offering expenses.