UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2024

☐ **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 001-40237

GAIN THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**85-1726310**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)
4800 Montgomery Lane, Suite 220 Bethesda, Maryland	**20814**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: **(301) 500-1556**

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

Title of Each Class	Trading Symbol	Name of Each Exchange on Which Registered
Common Stock, par value $0.0001 per share	GANX	Nasdaq Global 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 X

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No X

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 X 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 X No ☐

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

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

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 Act). Yes ☐ No X

The aggregate market value of the common equity held by non-affiliates of the Registrant on June 30, 2024 (the last business day of the Registrant's second fiscal quarter), based upon the closing price of $1.28 of the Registrant's common stock as reported on the Nasdaq Global Market, was approximately $30.8 million.

As of February 28, 2025, 27,786,952 shares of the registrant's Common Stock were outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

The information required by Part III of this Annual Report on Form 10-K, to the extent not set forth herein, is incorporated by reference from the Registrant's definitive proxy statement for the 2025 Annual Meeting of Stockholders, which will be filed with the Securities and Exchange Commission within 120 days of the Registrant's fiscal year ended December 31, 2024.

GAIN THERAPEUTICS, INC.
ANNUAL REPORT FORM 10-K

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Unless the context suggests otherwise, references in this Annual Report on Form 10-K, or the Annual Report, to "Gain," the "Company," "we," "us," and "our" refer to Gain Therapeutics, Inc. and, where appropriate, its wholly owned subsidiaries.

Magellan™ is our trademark. All other brand names and service marks, trademarks and other trade names appearing in this Annual Report are the property of their respective owners.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). These forward-looking statements can be identified by the fact that they do not relate strictly to historical or current facts and are often characterized by the use of words such as "aim", "believe," "can," "could," "potential," "plan," "predict," "goals," "seek," "should," "may," "may have," "would," "estimate," "continue," "anticipate," "intend," "expect" or the negative of these terms, other comparable terminology or by discussions of strategy, plans or intentions. These include, but are not limited to, statements about:

- our ability to continue as a going concern and our needs for additional financing;
- our ability to maintain compliance with the continued listing requirements of the Nasdaq Stock Market LLC ("Nasdaq");
- our ability to accurately estimate anticipated operating losses, expenses, future revenues, capital requirements, including our anticipated cash runway;
- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and our research and development programs;
- the success of our efforts to expand our pipeline of product candidates and develop marketable products through the use of our Magellan™ platform;
- our ability to develop, obtain regulatory approval for and commercialize our current and future product candidates;
- our expectations regarding collaborations and other agreements with third parties and their potential benefits;
- the timing of investigational new drug, or IND, submissions, initiation of preclinical studies and clinical trials, and timing of expected clinical results for our product candidates;
- our success in early preclinical studies, which may not be indicative of results obtained in later studies or clinical trials;
- the potential benefits of our product candidates;
- our ability to identify patients with the diseases treated by our product candidates, and to enroll healthy volunteers and patients in clinical trials;
- our ability to obtain, maintain and protect our intellectual property;
- our reliance upon intellectual property licensed from third parties, including the license to use certain components of the Magellan™ platform;
- our ability to identify, recruit and retain key personnel;
- developments or projections relating to our competitors or our industry;

- the impact of laws and regulations;
- our expectations regarding government and third-party payor coverage and reimbursement;
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups ("JOBS") Act;
- the impact of liquidity concerns at and failures of banks and other financial institutions, capital market instability, exchange rate fluctuations, supply chain disruptions and increases in commodity, energy and fuel prices;
- the impacts of pandemics or endemics on our operations, access to capital, research and development and clinical trials and potential disruption in the operations and business of third-party manufacturers, contract research organizations, other service providers, and collaborators with whom we conduct business;
- the impact of other global events, including political instability, natural disaster, events of terrorism and wars, including the war between Ukraine and Russia, and the corresponding tensions created from such conflict between Russia, the United States and countries in Europe as well as other countries such as China; and the conflict between Hamas and Israel; and
- other factors and assumptions described in this Annual Report.

You should read this Annual Report with the understanding that such forward-looking statements involve known and unknown risks, expectations, uncertainties, assumptions, estimates and projections about our company and other important factors that could cause our actual results, performance or achievements, actual industry results, or other actual results or events to differ materially from historical results, from any plans, intentions, or expectations disclosed in such forward-looking statements or from any future results, performance, achievements or other events expressed, suggested or implied by such forward-looking statements. Therefore, you should not rely on any forward-looking information or statements as predictors of future results or events. Factors that could cause or contribute to such differences in results and events include, without limitation, those specifically addressed under the headings "Risk Factors" and "Management's Discussion and Analysis of Financial Conditions and Results of Operations" in this Annual Report and in our subsequent filings with the Securities and Exchange Commission. The effect of these factors is difficult to predict. In addition, factors other than these could also adversely affect our results, and the reader should not consider these factors to be a complete set of all potential risks or uncertainties. New factors emerge from time to time, and management cannot assess the impact of any such factor on our business or the extent to which any factor, or combination of factors, may cause results or events to differ materially from those contained in any forward-looking statement.

Any forward-looking statements included herein speak only as of the date of this Annual Report, and we undertake no obligation to update any forward-looking information or statements for any reason after the date of this Annual Report to conform these statements to actual results or changes in expectations, except as required by law. All forward-looking statements attributable to us are expressly qualified by the foregoing cautionary statements.

PART I

ITEM 1. BUSINESS

Overview

We are a biotechnology company developing novel small molecule therapeutics to treat diseases across several therapeutic areas, including central nervous system ("CNS") disorders, lysosomal storage disorders ("LSDs"), metabolic disorders, and other diseases that can be targeted through protein degradation, such as oncology.

GT-02287 - our lead product candidate, for the treatment of Parkinson's disease, is currently being evaluated in a Phase 1b study in people with Parkinson's disease with or without a GBA1 mutation.

We have generated an extensive preclinical data package providing evidence of the mechanism of action and safety of GT-02287. In preclinical models of GBA1 and idiopathic Parkinson's disease, GT-02287 has been shown to restore glucocerebrosidase, or GCase, function in the lysosome, improve mitochondrial health, reduce toxic lipid substrates and toxic forms of alpha-synuclein, reduce neuroinflammation, improve survival of dopaminergic neurons, increase dopamine levels, completely restore locomotor function and improve cognition, and reduce plasma-based neurodegeneration maker, neurofilament light chain (NfL) back to the level of healthy animals.

In September 2024, we reported results from a first-in-human Phase 1 clinical trial completed in Australia to assess the safety, tolerability, pharmacokinetics, and food effect of GT-02287 in healthy participants. The study enrolled 72 healthy volunteers and included a single ascending dose part during which the study participants (n=40) received one dose of GT-02287 at different dose levels, and a multiple ascending dose part during which the study participants (n=32) received one daily dose of GT-02287 for 14 days at different dose levels. Review of the unblinded data after database lock confirmed that single and multiple doses of GT-02287 were safe and generally well tolerated up to and including the highest planned dose levels across all age groups (approximately 15% of which were over the age of 50 years).

The single and multiple dose levels tested were safe and generally well tolerated, with no serious adverse events or Grade 3 (severe) adverse events observed, and no other safety signals detected. The PK profile of GT-02287 was linear across the tested dose ranges, and plasma exposures at daily doses of 7.7 mg/kg and above were within the projected therapeutic range. GT-02287 was measurable in cerebrospinal fluid (CSF) at levels in line with rodent levels at effective doses from our preclinical models, demonstrating CNS exposure. Notably, GCase activity in dried blood spots increased approximately 53% in subjects who received 13.5 mg/kg GT-02287 (the highest dose cohort and the only dose cohort analyzed for GCase activity) but not in those who received placebo, demonstrating target engagement and modulation of GCase enzyme. GCase activity continued to increase 12 hours post-dose at 14 days, the furthest time point analyzed in the study. We believe these results support the continued development of GT-02287 and its potential as a biology-modifying treatment for Parkinson's disease. Importantly, the favorable safety and tolerability profile at oral dose levels that resulted in therapeutic plasma levels, CNS exposure, and target engagement further strengthens GT-02287's potential to be a lead treatment for Parkinson's disease in patients with or without a GBA1 mutation.

In December 2024, we received approval in Australia to initiate a Phase 1b trial for GT-02287 in people with Parkinson's disease with or without the GBA1 mutation. We are working with local Parkinson's disease (PD) advocacy groups in Australia to support enrollment and expect enrollment to complete in the summer of 2025 with interim data from the study expected by mid-2025. The Phase 1b open-label trial will assess the safety and tolerability of 13.5 mg/kg/day of GT-02287 for three months in patients with GBA1-PD or idiopathic Parkinson's disease. Secondary endpoints include pharmacokinetics, GCase modulation, levels of GCase substrates, and other biomarkers in plasma and cerebrospinal fluid. The primary goal of the Phase 1b trial is to assess the safety and tolerability of GT-02287. Upon successful completion we expect to begin planning a Phase 2 study during the second half of 2025.

In preparation for the treatment of Parkinson's disease patients, we initiated a chronic (6 months in rodents and 9 months in non-rodents) preclinical toxicity study in July 2024, enabling the conduct of clinical studies in patients with a GT-02287 treatment duration beyond three months. The 6- and 9-month studies will be completed in the second quarter of 2025 and the third quarter of 2025, respectively.

Our Magellan™ Platform

We use our computational target and drug discovery platform, Magellan™, to discover novel allosteric binding sites on proteins implicated in a disease and to identify proprietary small molecules that bind these sites to modulate protein function and treat the underlying cause of the disease. We believe that Magellan™ is uniquely suited to identify allosteric binding sites on the protein surface, which are different from the active (or orthosteric) binding site where the natural ligand of the protein binds. Targeting an allosteric binding site instead of the active binding site of a protein provides numerous advantages, including: the ability to regulate proteins implicated in disease through several different mechanisms of action covering both functional and conformational effects, including stabilization, destabilization, targeted degradation, allosteric inhibition, and allosteric activation of the targeted protein; improved specificity of small molecules because binding to an allosteric binding site is non-competitive with the natural substrate that binds to the active binding site; and the ability to identify small molecules with more favorable drug-like properties. Discovering and targeting novel allosteric sites with our platform not only reduces traditional drug discovery timelines but enables rational drug design and offers the potential for superior small molecule drugs that are highly specific and that can penetrate hard to reach tissues and cross the blood-brain barrier.

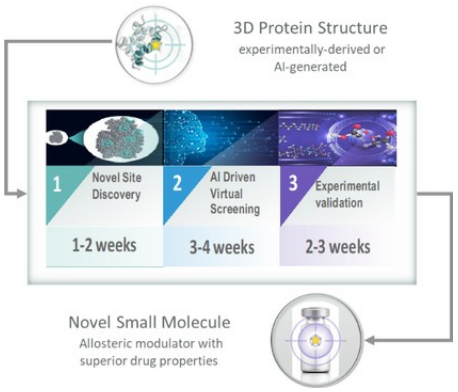
Our Research Programs

We have used the Magellan™ drug discovery platform to identify novel allosteric sites and small molecules for all of our pipeline programs. We plan to continue to advance our existing research programs and initiate additional programs targeting allosteric binding sites identified with the Magellan™ platform in various therapeutic areas through academic partnerships, co-development and licensing arrangements.

Our Platform for Computational Target and Drug Discovery

Overview

A majority of disease-causing proteins (up to 90%) cannot be targeted due to the lack of a known binding site. Our Magellan™ platform was designed to address this problem. We use the platform to discover novel binding sites on proteins implicated in a disease and to identify proprietary small molecules that bind these sites to modulate protein function and treat the underlying cause of the disease. We focus specifically on allosteric binding sites distinct from the protein's active, or orthosteric, binding site, where a small molecule can attach and trigger an effect that may lead to a therapeutic benefit. We refer to the small molecules we identify that bind to these allosteric sites as structurally targeted allosteric regulators, or STARs, to reflect their mechanism of action and how they are discovered. The graphic below provides an overview of Magellan™.



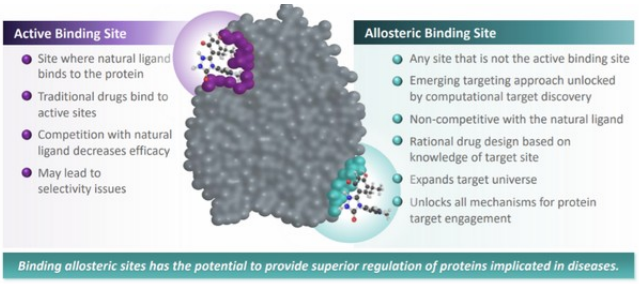
Allosteric Binding Site Identification

Using the three-dimensional structure of proteins that have been experimentally derived or generated or predictive protein structures from AI-powered databases such as Alphafold, our Magellan™ platform applies various computational methods and proprietary algorithms to identify and map previously uncharacterized clusters of binding

hotspots on the protein surface where a small molecule can potentially bind. The number, density, nature and quality of these hotspot clusters determine the druggability of the protein, which refers to whether drug-like small molecules can effectively bind to the particular site on the target protein with an appropriate potency.

Advantages of Targeting Allosteric Binding Sites

We focus on allosteric binding sites, which offer a number of advantages compared to targeting the active binding site of a protein, including the ability to regulate proteins implicated in disease through several different mechanisms of action covering both functional and conformational effects, improved specificity of small molecules because binding to an allosteric binding site is non-competitive with the natural ligand that binds to the active binding site, and the ability to identify small molecules with more favorable drug-like properties. The graphic below provides an overview of the differences and benefits of allosteric binding sites compared to active binding sites.



Identification of Structurally Targeted Allosteric Regulators ("STARs") – Our Molecular Hypothesis

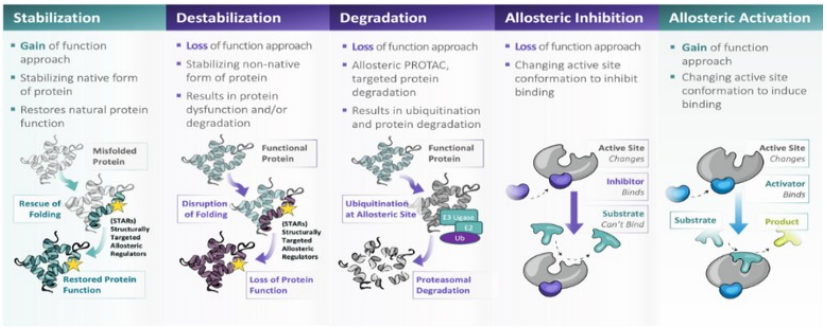
After an allosteric site has been identified, characterized and selected for targeting, we then use our proprietary structure-based virtual screening methodology to explore virtual chemical libraries of over 50 billion compounds to identify those that may bind to the hotspot and have a functional effect. Using this information, we develop structural templates to guide the development of a narrowed pool of unique and proprietary small molecules that bind to the newly discovered allosteric sites.

We believe our process for identifying STARs provides several advantages over traditional drug discovery approaches such as random high-throughput screening. In high-throughput screening, very large libraries of randomly selected molecules are tested for their ability to perform a specific function such as binding to a target protein. This approach typically results in a large number of positive hits that must then be laboriously analyzed to identify compounds with relevant properties and effect. A high-throughput screening campaign may take up to two years or more to complete, and, on average, only 0.1% of all compounds tested in this manner bind to the targeted protein with the desired effect. In contrast, our approach is significantly less expensive, significantly faster and significantly more effective. We run our Magellan™ simulations for target and drug discovery in supercomputer centers where we pay only for time used as and when needed. We can identify a novel allosteric site in one to two weeks, set up and complete virtual screening in three to four weeks, and validate compounds experimentally in two to three weeks. Our average success rate for experimentally validated compounds is 14%, a greater than 100-fold higher success rate compared to traditional high throughput screening methods. Further, every small molecule hit identified by our platform is experimentally tested based on a two-part molecular hypothesis to confirm that (1) the compound has a positive effect on the relevant biomarkers implicated in the disease and (2) binding to the allosteric binding site identified with the Magellan™ platform has the intended effect on protein function.

Allosteric Regulators Cover Several Mechanisms of Action

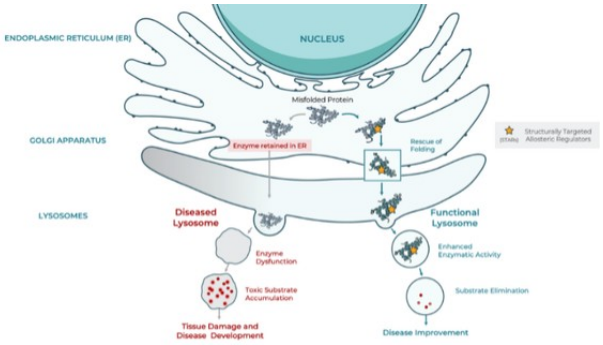
Another benefit of targeting allosteric sites is that it allows for several different mechanisms of action. In our LSD and Parkinson's disease programs, we have identified STARs that are designed to bind to a protein with a tendency to misfold, stabilize that protein in its correctly folded state and restore protein function. However, in areas such as oncology, we have identified STARs that are designed to destabilize target proteins by binding to a non-native or mutant form of the protein and render it inactive. There are several additional potential mechanisms of action including allosteric targeted protein degradation, as well as traditional allosteric inhibition or activation by inducing a

conformational change to inhibit or induce binding by the natural ligand of the active site of the protein. The graphic below provides an overview of the different mechanisms of action available through allosteric binding sites.



Enzyme Misfolding and Disease

Proteins are large biomolecules that have a vast array of functions in different cell types in the body. Enzymes are a type of protein that accelerate and facilitate chemical reactions inside of cells by acting on substrates and converting those substrates into different chemical products. To perform their function in the body, enzymes and other proteins must be folded into the correct three-dimensional shape. Misfolded enzymes may not function properly, which can lead to the toxic accumulation of unprocessed substrate which is the cause of many rare genetic diseases, including LSDs and some neurodegenerative diseases such as certain forms of Parkinson's disease. Enzyme misfolding may arise from genetic mutations that disrupt the folding pattern as well as from cellular stress due to aging and inflammation. Therapeutic small molecules that facilitate the folding of enzymes into their correct shape can restore function and the proper processing of substrate. As illustrated below, in genetic diseases caused by protein misfolding and dysfunction, such as LSDs or GBA1 Parkinson's disease, the gene that codes for an enzyme is mutated and expresses a misfolded enzyme. The misfolded enzyme cannot traffic through the cell resulting in toxic substrate accumulation in the lysosome. We believe that our STARs will have the ability to bind to the allosteric site of the defective enzyme and restore wild type activity and thus serve as potential therapeutic treatments for diseases. The graphic below provides an overview of the postulated mechanism of action.



Limitations of Current Therapies for the Treatment of LSDs

Current therapeutic approaches to address misfolded enzymes have inherent limitations. Drugs that bind to the active site of the enzyme or other target proteins impair the protein's function to some degree by competing with the active substrate, decreasing efficacy and potentially leading to selectivity issues. Other treatments such as enzyme replacement therapy, or ERT, in which new functional enzymes are infused into the patient, are not suitable for treating neurological conditions because currently available ERTs cannot cross the blood-brain barrier. Gene therapy, which aims to replace mutated genes with non-mutated genes that then can express functional enzymes, is not readily accepted for treating neurological conditions because the procedure is invasive in nature and the efficacy of treating

neurological conditions remains to be established. In addition, clinical development, manufacturing and commercialization of gene therapies remains challenging in light of safety risks, complex manufacturing processes and high production costs, and difficulties in establishing prices acceptable to payors and health care systems. Given these limitations on current therapies and novel therapeutics approaches, we believe patients would benefit from small molecules acting as structurally targeted allosteric regulators that offer a new therapeutic approach both on their own and, potentially, in combination with existing therapies. We believe our therapeutic approach represents a potentially significant change from current approaches by addressing protein misfolding using our efficient and proprietary ability to identify previously undiscovered allosteric sites and compounds that avoid the active sites of enzymes and cross the blood-brain barrier or penetrate other hard-to-treat tissues such as bone and cartilage.

Our Pipeline of STARs

We are leveraging our Magellan™ technology platform to develop a pipeline of novel small molecule drug candidates to address complex diseases. The platform is disease agnostic and provides us with the ability to expand our pipeline, quickly, efficiently and at low cost. We are currently focusing on progressing our clinical-stage lead program in Parkinson's disease. In addition, we plan to continue to advance our existing research programs and initiate additional programs targeting allosteric binding sites identified with the Magellan™ platform in various therapeutic areas through discovery collaborations with industry partners and academic institutions.

Our Product Pipeline

GCase Enzyme-Related Neurodegenerative Diseases and LSDs

GCase is a lysosomal enzyme encoded by the GBA1 gene that is needed to break down fatty substrates, in particular glucosylceramide and glucosylsphingosine, into sugar and fat. If GCase is dysfunctional or not available at sufficient levels, these fatty substrates start to accumulate and eventually become directly toxic to the cells, as well as initiating a disease cascade including lysosomal and mitochondrial dysfunction and accumulation of aggregated proteins associated with neurodegenerative diseases such as alpha synuclein and phospho-tau leading to neuroinflammation and neuronal cell death. Our clinical-stage lead product candidate GT-02287 has been shown to restore enzymatic function of GCase in the lysosome, which may be useful as a treatment for Parkinson's disease, or PD, Gaucher disease, oGD, an LSD, and other neurodegenerative diseases, including dementia with Lewy bodies and Alzheimer's disease.

Overview of GBA1 Parkinson's Disease

GBA1 Parkinson's disease is associated with heterozygous mutations in the GBA1 gene, which leads to the expression of misfolded and dysfunctional GCase. It is widely accepted that GCase deficiency has a biological role as a modifier or facilitator of Parkinson's disease pathogenesis in the brain. Brain autopsy studies have shown that decreased levels of GCase are also found in patients with idiopathic Parkinson's disease (without GBA1 mutations). Reduced GCase activity may enhance the risk for Parkinson's disease by facilitating a pathological hallmark, namely aggregated alpha-synuclein accumulation. Aggregated alpha-synuclein accumulation and GCase deficiency are thought to act in a debilitating cycle. GCase deficiency can cause the accumulation of glucosylceramide and glucosylsphingosine substrates, which has been reported to directly affect the accumulation and aggregation of alpha-

synuclein. In addition, increased aggregated alpha-synuclein levels can lead to less GCase activity, which in turn can lead to more aggregated alpha-synuclein accumulation.

Parkinson's disease is reported to affect more than ten million people worldwide. Around 10% of patients with Parkinson's disease carry GBA1 mutations, making it the largest genetic risk factor for the disease. At present, there is no effective cure for Parkinson's disease. Current approved therapies for Parkinson's disease are limited to symptomatic treatments such as levodopa, dopaminergic receptor agonists and inhibitors of enzymes related to dopamine metabolism such as monoamine oxidase inhibitors and catechol-O-methyltransferase inhibitors. These therapies aim to improve overall dopaminergic function. The benefits of these types of treatments diminish over time as the disease progresses, and these therapies do not impact the non-motor symptoms such as cognitive decline or the progression of the disease. As the disease progresses, the non-motor symptoms, such as cognitive impairment and dementia, can lead to severe morbidity and mortality.

Overview of Gaucher Disease

Gaucher disease is an inherited LSD caused by homozygous mutations of the GBA1 gene that result in the misfolding and subsequent dysfunction of GCase. Gaucher disease is traditionally classified according to one of three types. Type 1 Gaucher disease is traditionally referred to as a non-neuronopathic form of the disease, for which some treatments are available, but evolving science has shown that patients with type 1 Gaucher disease may also manifest neurological symptoms later in life. Current ERT and gene therapy treatments are unable to address the onset of type 1 neurological symptoms because these treatments are unable to cross the blood-brain barrier. Unlike Gaucher disease type 1, Gaucher disease types 2 and 3 have early onset brain degeneration that worsens over time. For this reason, Gaucher disease types 2 and 3 are known as neuronopathic Gaucher disease (nGD). Currently, there is no effective treatment for nGD. In type 2 Gaucher disease, there is neurological impairment that presents before birth through the first months of life, progresses rapidly, and is typically fatal within two years. It is a devastating disorder characterized by neurodegeneration and brainstem dysfunction. Additionally, infants with Gaucher disease may have abnormally large organs, deficiency in growth, seizures and compromised swallow and airway problems. Gaucher disease type 3 (also known as chronic neuronopathic Gaucher disease) has a later and more gradual onset compared with type 2. People with Gaucher disease type 3 may survive into adulthood with a wide variety of signs and symptoms, including seizures, skeletal irregularities, eye movement disorders, cognitive and coordination problems as well as enlarged liver and spleen, respiratory problems, and blood disorders.

Loss of GCase activity can cause the buildup of glucosylceramide and glucosylsphingosine in the lysosomes of macrophages, and the accumulation of these lipid substrates in neuronal cells can result in neurological symptoms.

The prevalence of Gaucher disease type 1 (non-neuronopathic Gaucher disease) is reported as 1:57,000 to 75,000 people worldwide. Type 1 is the most common form in Western countries (around 95%). The prevalence of type 2 and type 3 Gaucher disease, or nGD, is approximately 1:100,000 people worldwide, and these forms are the most common in non-Western countries, especially in Asian countries where they make up more than 50% of the Gaucher disease patient population. At present there are no available treatment options for neuronopathic Gaucher disease, but ERT is still used to address organ enlargement, hematological manifestation, and bone disease, as well as to improve the quality of life for these patients. ERT does not cross the blood-brain barrier and is not efficient in treating neurological manifestations, therefore creating a significant unmet medical need in this patient population.

Overview of Dementia with Lewy Bodies and Alzheimer's Disease

Dementia with Lewy bodies (DLB) and Alzheimer's disease are both dementia types that lead to decline in memory, thinking and behavior. In DLB, deposits of the protein alpha-synuclein aggregate in neuronal cells and form inclusions called Lewy bodies. GT-02287 has been shown to reduce alpha-synuclein aggregation and to improve cognitive ability in preclinical models.

In Alzheimer's disease, the main pathophysiology involves the development of amyloid plaques and neurofibrillary tangles which contain aggregated amyloid beta and Tau protein, respectively. Lysosomal dysfunction has been shown to play a role in aggregation of Tau and development of amyloid plaques. GT-02287 has been shown to reduce tau hyperphosphorylation in response to aggregated amyloid-beta and neuronal cell death in response to aggregated tau in preclinical models.

Preclinical Characterization of Lead Compound GT-02287 for the Treatment of GBA1-Related Diseases

We have assessed the effect of our lead product candidate GT-02287 in various cell-based and animal models of PD and GD.

Activity in Biophysical and Cell-based Assays

Biophysical assay results have demonstrated that GT-02287 binds to the GCase protein and increases its thermal stability. In cell-based functional assays, we observed a dose-dependent increase in GCase activity in normal and GCase mutant cells when treated with GT-02287 as well as a concomitant depletion of the GCase substrates glucosylceramide and glucosylsphingosine. GT-02287 also has shown GCase enzyme enhancement in an extended panel of patient-derived cells representative of the most frequent and pathogenic GBA1 mutations related to GBA1 PD and nGD. In addition, we reported that GT-02287 increased GCase enzyme levels, co-localization of GCase with lysosomes, reduced GlcCer accumulation as well as phosphorylated and aggregated alpha-synuclein accumulation in neurons derived from patients carrying GBA1 mutations.

Pharmacokinetics

Studies in mice, rats and dogs have shown that GT-02287 is quickly absorbed following oral administration, reaching the maximal concentration in plasma (Tmax) between 0.5 and 2 hours with a plasmatic half-life (t1/2) of about 2 hours in mice, 3 hours in rats and 5 hours in dogs.

We examined GT-02287 in neuro-PK studies to evaluate its brain penetration properties, and we observed high brain exposure with a brain-to-plasma ratio level greater than one.

In-Vivo Pharmacology in PD Animal Models

Mice were administered low levels of irreversible GCase inhibitor CBE and received an injection of alpha-synuclein preformed fibrils (PFFs) directly into the brain to mimic reduced GCase activity and alpha-synuclein accumulation seen in GBA -PD, or PFFs only to mimic idiopathic Parkinson's disease. GT-02287, orally administered once a day after the mice has started to show deficits in motor function, showed statistically significant improvements in GCase activity, restoration of markers of lysosomal and mitochondrial integrity, reduction of aggregated alpha-synuclein, reduced neuroinflammation, increased survival of dopaminergic neurons in the substantia nigra, increased dopamine levels in the striatum, increased survival of cortical neurons, and decreased levels of NfL (neurofilament light chain), an emerging neurodegeneration biomarker, in the plasma, in both the GBA1 and idiopathic PD models. These effects on disease markers were accompanied by a dose-dependent behavioral effect shown by improved neuromotor strength as measured by the wire hang test and increased coordination as measured by the beam walk test as well as an improvement in nest building which is a complex behavior involving an element of cognitive function. Withdrawal of the compound resulted in a reduction in GCase activity as expected but improvement in disease markers and on motor and cognitive function were maintained for at least 9 days, suggesting a disease slowing effect of GT-02287.

Nonclinical Toxicology and Safety Studies

The sub-chronic GLP general toxicity studies in both rodents and non-rodents were successfully completed in the second quarter of 2024, without any delays, which allows for the treatment of patients over a three-month period. To further advance our clinical development plan for GT-02287, we initiated a chronic toxicity study—lasting six months in rodents and nine months in non-rodents during the second half of 2024. This will enable clinical studies involving GT-02287 in patients for treatment durations exceeding twelve months. The in-vivo phase of the chronic toxicity study in rodents was completed in February 2025, and the final quality assurance audited report is anticipated by May 2025. The completion of the in-vivo phase in non-rodent species is expected by the second quarter of 2025. Additionally, a comprehensive mass-balance study using radiolabeled GT-02287 in rats was concluded in 2024, demonstrating the distribution of the compound within the central nervous system.

Clinical Study

In September 2023, we initiated the first-in-human Phase 1 clinical trial to assess the safety, tolerability, pharmacokinetics, and food effect of GT-02287 in healthy participants. The study design included a single ascending

dose part during which the study participants receive one dose of GT-02287 at different dose levels, and a multiple ascending dose part during which the study participants received one daily dose of GT-02287 for 14 days at different dose levels. The study was completed in July 2024 and the quality assurance audited interim report was finalized in the third quarter of 2024. In the ongoing Phase 1b study, participants with Parkinson's disease will receive GT-02287 for a period of three months to evaluate safety, tolerability, pharmacokinetics, and the effect of GT-02287 on certain biomarkers of the GCase disease cascade

Pipeline Programs in Research and Discovery Phases

In addition to our clinical-stage lead program for Parkinson's disease, we are planning to progress additional programs in the research and discovery phases in conjunction with academic and industry collaboration.

Competition

The biotechnology and pharmaceutical industries are characterized by the rapid evolution of technologies and understanding of disease etiology, intense competition, and a strong emphasis on intellectual property. We believe that our Magellan™ platform, our scientific capabilities, know-how and experience provide us with competitive advantages. However, we expect substantial competition from multiple sources, including major pharmaceutical, specialty pharmaceutical, and existing or emerging biotechnology companies, academic research institutions and governmental agencies and public and private research institutions worldwide. Many of our competitors, either alone or with their collaborations, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient enrollment in clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. As a result, our competitors may discover, develop, license, or commercialize products before or more successfully than we do. We are not aware of any other companies that are taking the same therapeutic approach to protein folding disorders similar to the ones we are pursuing. However, we are aware of companies developing products for the same target indications. For example, companies targeting GBA-PD using small molecules include Vanqua Bio, BIAL, and Caraway Therapeutics. While all of these approaches are small molecules hypothesized to increase GCase levels, they differ from our approach because our molecules act as non-competitive pharmacological chaperones, specifically focused on stabilizing and restoring lysosomal function to misfolded GCase. There are also companies targeting GBA-PD through other modalities such as gene therapies. These companies include, among others: Prevail Therapeutics, which is evaluating a potential gene therapy candidate in a Phase 1/2 clinical trial, and Voyager Therapeutics.

Additionally, new or advanced technologies developed by our competitors may render our current or future product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

Strategic Transactions; Collaboration and Licensing Arrangements

In connection with our business development activities, we enter into collaborative and licensing arrangement with third parties to use our Magellan™ drug discovery platform to discover novel allosteric sites on proteins and identify proprietary small molecules that bind these sites to disrupt or restore protein function as may be needed to treat a particular disease. We expect to continue to identify and evaluate collaboration, co-development and licensing opportunities that may be similar to or different from the collaborations and licenses that we have entered into.

Minoryx Therapeutics, S.L.

We have entered into a license agreement, dated December 20, 2017 (the "Minoryx License Agreement"), with Minoryx Therapeutics, S.L., a company organized under the laws of Spain ("Minoryx"), pursuant to which we obtained exclusive worldwide license rights from Minoryx to use and exploit its intellectual property ("IP"), including certain components of its computational drug discovery platform Magellan™ for the identification of non-competitive pharmacological chaperones and exclusive worldwide sublicense rights to certain IP licensed by Minoryx from the University of Barcelona and the Institució Catalana de Recerca i Estudis Avançats. Under the terms of the Minoryx License Agreement, we have an exclusive, worldwide, royalty-bearing, assignable, transferable license, including the right to license through multiple tiers of sublicense, to Minoryx's IP to make, have made, use, import, export, offer to

sell, have sold, copy, modify, perform, display, create derivative versions of products in the licensed field or otherwise to exploit Minoryx's IP in the field. Minoryx's IP includes certain components of the Magellan™ drug discovery platform, certain proprietary Minoryx compounds acting as pharmacological chaperones, all patents and pending applications related thereto and Minoryx's Know-How. We also have an exclusive, worldwide, royalty-bearing, assignable, transferable sublicense, including the right to sublicense through multiple tiers of sublicense, to the IP of Universitat de Barcelona (UB) and Institucio Catalana de Recerca i Estudis Avancats (ICREA) in EP11380102 and know-how and software related thereto, for the purpose of making, having made, using, importing, offering to sell, selling and having sold, copying, modifying, performing, displaying, and creating derivative versions of products in the field. Under the Minoryx License Agreement, products include any product in the field that would infringe the UB/ICREA IP or the Minoryx IP in the absence of the license provided therein. Also, the field encompasses any field of use and commercialization of the UB/ICREA IP or the Minoryx IP. Unless earlier terminated, the Minoryx License Agreement expires upon expiration of the royalty term, which occurs ten years after the first product covered by the licensed IP is commercialized. Khalid Islam, the Chairman of our board of directors and one of our founders, is currently the Chairman of the board of directors of Minoryx.

As consideration for the license grant from Minoryx, we have agreed to pay Minoryx royalties on a product-by-product basis based on the licensed IP used by us, ranging from a high single digit to low single digit percentage of net revenues of products during the royalty term commencing on the effective date of the Minoryx License Agreement and continuing until the 10th anniversary of the first product commercialization. Upon the expiration of the royalty term for a product or service in a country, the license with respect to the product or service, as the case may be, shall become royalty-free, fully-paid, irrevocable and perpetual.

The Minoryx License Agreement will terminate upon expiration of the royalty term (which is the 10th anniversary of the commercialization of the first product covered by the licensed IP) or by mutual agreement. In addition, each party has the right to terminate the Minoryx License Agreement upon a material breach by the other party that remains uncured. Minoryx has the right to terminate the Minoryx License Agreement on a country-by-country basis if we abandon the technology or use the technology for purposes in violation of law and we fail to cure such abandonment or unlawful use. We may terminate the Minoryx License Agreement at any time upon 90 days written notice.

Intellectual Property

We strive to protect and enhance the proprietary technologies, inventions and improvements that we believe are important to our business, including seeking, maintaining and defending patent rights, whether developed internally or licensed from third parties. Our policy is to seek to protect our proprietary position by, among other methods, pursuing and obtaining patent protection in the United States and in jurisdictions outside of the United States related to our proprietary technology, inventions, improvements, platforms and our product candidates that are important to the development and implementation of our business.

As of February 2025, our patent portfolio consisted of two patents granted in the U.S., Europe, and Japan. three international PCT applications in national phase stage, two international PCT applications published in 2024 and entering national phase in 2025, and two international PCT applications to be published in 2025.

In regard to our computational drug discovery platform Magellan™, we in-licensed the European patent under the Minoryx License Agreement, which is owned by UB/ICREA and has claims directed to a method of binding site and binding energy determination by mixed explicit solvent simulations. This patent is expected to expire in 2032.

In regard to our GLB program, we in-licensed from Minoryx pursuant to the Minoryx License Agreement, a patent family granted in U.S., Europe and Japan with claims directed to composition of matter. These patent applications are expected to expire in 2037, not giving effect to any potential patent term extensions and patent term adjustments and assuming payment of all appropriate maintenance, renewal, annuity or other governmental fees.

In regard to our GBA program, we in-licensed from Minoryx pursuant to the Minoryx License Agreement, a patent family granted in U.S., Europe and Japan with claims directed to composition of matter. These patent applications are expected to expire in 2037, not giving effect to any potential patent term extensions and patent term adjustments and assuming payment of all appropriate maintenance, renewal, annuity or other governmental fees.

Individual patents extend for varying periods depending on the date of filing of the patent application or the date of patent issuance and the legal term of patents in the countries in which they are obtained. Generally, patents issued for regularly filed applications in the United States are granted a term of 20 years from the earliest effective non-provisional filing date or the filing date of a PCT application that designates the United States. In addition, in certain instances, a patent term can be extended to recapture a portion of the U.S. Patent and Trademark Office, or USPTO, delay in issuing the patent as well as a portion of the term effectively lost as a result of the FDA regulatory review period. However, as to the FDA component, the restoration period cannot be longer than five years and the total patent term including the restoration period must not exceed 14 years following FDA approval. The duration of foreign patents varies in accordance with provisions of applicable local law,but typically is also 20 years from the earliest effective filing date, which is typically the filing date of the PCT application. However, the actual protection afforded by a patent varies on a product-by-product basis, from country to country and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

Furthermore, we rely upon trade secrets and know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, using confidentiality agreements with our collaborators, employees and consultants and invention assignment agreements with our employees. We also have confidentiality agreements or invention assignment agreements with our collaborators and selected consultants. These agreements are designed to protect our proprietary information and, in the case of the invention assignment agreements, to grant us ownership of technologies that are developed through a relationship with a third party. These agreements may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our collaborators, employees and consultants use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Our success will also depend in part on not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, or our drugs or processes, obtain licenses or cease certain activities. Our breach of any license agreements or failure to obtain a license to proprietary rights that we may require to develop or commercialize our future drugs may have an adverse impact on us. If third parties have prepared and filed patent applications prior to March 16, 2013 in the United States that also claim technology to which we have rights, we may have to participate in interference proceedings in the USPTO to determine priority of invention.

Government Regulation

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of drugs. We, along with our vendors, contract research organizations and contract manufacturers, will be required to navigate the various preclinical, clinical, manufacturing and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval of our product candidates. The process of obtaining regulatory approvals of drugs and ensuring subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources.

In the U.S., the FDA regulates drug products under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, as amended, its implementing regulations and other laws. If we fail to comply with applicable FDA or other requirements at any time with respect to product development, clinical testing, approval or any other legal requirements relating to product manufacture, processing, handling, storage, quality control, safety, marketing, advertising, promotion, packaging, labeling, export, import, distribution, or sale, we may become subject to administrative or judicial sanctions or other legal consequences. These sanctions or consequences could include, among other things, the FDA's refusal to approve pending applications, issuance of clinical holds for ongoing studies, withdrawal of approvals, warning or untitled letters, product withdrawals or recalls, product seizures, relabeling or repackaging, total or partial suspensions of manufacturing or distribution, injunctions, fines, civil penalties or criminal prosecution.

The process required by the FDA before our product candidates are approved as drugs for therapeutic indications and may be marketed in the U.S. generally involves the following:

- completion of extensive non-clinical studies in accordance with applicable regulations, including studies conducted in accordance with good laboratory practice, or GLP, requirements;
- submission to the FDA of an Investigative New Drug, or IND, application, which must become effective before clinical trials may begin;
- approval by an institutional review board, or IRB, or independent ethics committee at each clinical trial site before each trial may be initiated;
- performance of adequate and well-controlled clinical trials in accordance with applicable IND regulations, good clinical practice, or GCP, requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- submission to the FDA of an NDA;
- a determination by the FDA within 60 days of its receipt of an NDA, to accept the filing for review;
- satisfactory completion of one or more FDA pre-approval inspections of the manufacturing facility or facilities where the drug will be produced to assess compliance with cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- potential FDA audit of the clinical trial sites that generated the data in support of the NDA;
- payment of user fees for FDA review of the NDA; and
- FDA review and approval of the NDA, including consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the drug in the U.S.

Preclinical Studies and Clinical Trials for Drugs

Before testing any drug in humans, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluations of drug chemistry, formulation and stability, as well as in vitro and animal studies to assess safety, and in some cases, to establish the rationale for therapeutic use. The conduct of preclinical studies is subject to federal and state regulations and requirements, including GLP requirements for safety/toxicology studies. The results of the preclinical studies, together with manufacturing information and analytical data, must be submitted to the FDA as part of an IND application. An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before clinical trials may begin. Some long-term preclinical testing may continue after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises concerns or questions about the conduct of the clinical trial, including concerns that human research patients will be exposed to unreasonable health risks, and imposes a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Submission of an IND may result in the FDA not allowing clinical trials to commence or not allowing clinical trials to commence on the terms originally specified in the IND.

The clinical stage of development involves the administration of the product candidate to healthy volunteers or patients under the supervision of qualified investigators. These investigators are generally physicians not employed by or under the trial sponsor's control, in accordance with GCP requirements, which also includes the requirement that all research patients provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters and criteria to be used in monitoring safety and evaluating effectiveness. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Furthermore, each clinical trial must be reviewed and approved by an IRB for each institution at which the clinical trial will be conducted to ensure that the risks to individuals participating in the clinical trial are minimized and are reasonable in relation to the anticipated benefits. The IRB also approves 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

completed. The FDA, the IRB or the sponsor may suspend or discontinue a clinical trial at any time on various grounds, including a finding that the patients are being exposed to an unacceptable health risk. There also are requirements governing the reporting of ongoing clinical trials and completed clinical trials to public registries. Information about clinical trials, including clinical trials results, must be submitted within specific timeframes for publication on the www.clinicaltrials.gov website.

A sponsor who wishes to conduct a clinical trial outside of the U.S. may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, the sponsor can submit data from the clinical trial to the FDA in support of an NDA. The FDA will accept a well-designed and well-conducted foreign clinical trial not conducted under an IND if the trial was conducted in accordance with GCP requirements and the FDA is able to validate the data through an onsite inspection, if deemed necessary.

Clinical trials to evaluate therapeutic indications to support NDAs for marketing approval are typically conducted in three sequential phases, which may overlap:

- *Phase 1*—Phase 1 clinical trials involve initial introduction of the investigational product into healthy human volunteers or patients with the target disease or condition. These studies are typically designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, excretion of the side effects associated with increasing doses, and, if possible, to gain early evidence of effectiveness.

- *Phase 2*—Phase 2 clinical trials typically involve administration of the investigational product to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.

- *Phase 3*—Phase 3 clinical trials typically involve administration of the investigational product to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval and physician labeling.

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 and are commonly intended to generate additional safety data regarding use of the product in a clinical setting. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

Progress reports detailing the results of the clinical trials, among other information, must be submitted at least annually to the FDA. Written IND safety reports must be submitted to the FDA and the investigators fifteen days after the trial sponsor determines that the information qualifies for reporting for serious and unexpected suspected adverse events, findings from other studies or animal or in vitro testing that suggest a significant risk for human volunteers and any clinically important increase in the severity or rate of a serious suspected adverse reaction over that listed in the investigator brochure. The sponsor must also notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction as soon as possible but in no case later than seven calendar days after the sponsor's initial receipt of the information.

Concurrent with clinical trials, companies usually complete additional laboratory and animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing the drug product in commercial quantities in accordance with cGMP requirements.

The manufacturing process must be capable of consistently producing quality batches of the product candidate. Manufacturers must develop, among other things, methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Special Protocol Assessment

The FD&C Act directs the FDA to meet with sponsors, pursuant to a sponsor's written request, for the purpose of reaching an agreement on the design and size of clinical trials intended to form the primary basis of an efficacy claim in an NDA or a Biologic Licensing Application (BLA). If an agreement is reached, the FDA will reduce the agreement to writing and make it part of the administrative record. This agreement is called a special protocol assessment (SPA). While the FDA's guidance on SPAs states that documented SPAs should be considered binding on the review division, the FDA has latitude to change its assessment if certain exceptions apply. Exceptions include public health concerns emerging that were unrecognized at the time of the protocol assessment, identification of a substantial scientific issue essential to the safety or efficacy testing that later comes to light, a sponsor's failure to follow the protocol agreed upon or the FDA's reliance on data, assumptions or information that are determined to be wrong.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including biological products, are required to register and disclose certain clinical trial information on the website www.clinicaltrials.gov. Information related to the product, patient population, phase of investigation, trial sites and investigators and other aspects of a clinical trial are then made public as part of the registration. Sponsors are also obligated to disclose the results of their clinical trials after completion. Disclosure of the results of clinical trials can be delayed in certain circumstances for up to two years after the date of completion of the trial. Competitors may use this publicly available information to gain knowledge regarding the progress of clinical development programs as well as clinical trial design.

U.S. Marketing Approval for Drugs

Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, 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. An NDA must contain proof of the drug's safety and efficacy. The marketing application may include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources. These sources include studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of the FDA. FDA approval of an NDA must be obtained before a drug may be marketed in the U.S.

The FDA reviews all submitted NDAs before it accepts them for filing and may request additional information rather than accepting the NDA for filing. The FDA must make a decision on accepting an NDA for filing within 60 days of receipt. Such decision could include a refusal to file by the FDA. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the NDA. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single trial may be sufficient in some instances, including (i) where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible or (ii) when there is one adequate and well-controlled clinical investigation plus other confirmatory evidence. Under the goals and polices agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA targets ten months from the filing date to complete its initial review of a new molecular entity NDA and respond to the applicant and six months from the filing date of a new molecular entity NDA for priority review. 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.

Further, under PDUFA, as amended, each NDA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. Fee waivers or reductions are available in certain circumstances including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs for products designated as orphan drugs unless the product also includes a non-orphan indication.

The FDA also may require submission of a Risk Evaluation and Mitigation Strategy, or REMS, program to ensure that the benefits of the drug outweigh its risks. The REMS program could include medication guides, physician communication plans, assessment plans and/or elements to assure safe use, such as restricted distribution methods, patient registries or other risk-minimization tools.

The FDA may refer an application for a novel drug 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.

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 and other requirements and the integrity of the clinical data submitted to the FDA as well as one or more sites where non-clinical testing was conducted to assure compliance with GLP and other 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 the FDA to reconsider 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, depending on the specific risk(s) to be addressed, 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. These other conditions include distribution and use restrictions or other risk management mechanisms under a REMS that 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 of 1983, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, a disease or condition that affects fewer than 200,000 individuals in the U.S., or if it affects more than 200,000 individuals in the U.S., there is no reasonable expectation that the cost of developing and making the product available in the U.S. for the disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA. Orphan designation does not convey any advantage in or shorten the duration of the regulatory review and approval process though companies developing orphan products are eligible for certain incentives such as tax credits for qualified clinical testing and waiver of application fees.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity during which the FDA may not approve any other applications to market the same therapeutic agent for the same indication, except in limited circumstances such as a subsequent product's showing of clinical superiority over the product with orphan exclusivity or where the original applicant cannot produce sufficient quantities of product. Competitors, however, may receive approval of different therapeutic agents for the indication for which the orphan product has exclusivity or obtain approval for the same therapeutic agent for a different indication than that for which the orphan product has exclusivity. Orphan product exclusivity could block the approval of one of our products for

seven years if a competitor obtains approval for the same therapeutic agent for the same indication before we do unless we are able to demonstrate that our product is clinically superior. If an orphan designated product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity. Further, orphan drug exclusive marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Expedited Development and Review Programs for Drugs

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 and Accelerated Approval. 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.

A new drug is eligible for Fast Track designation if it is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address unmet medical needs for such disease or condition. 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.

In addition, a new drug may be eligible for Breakthrough Therapy designation if it is intended to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints such as substantial treatment effects observed early in clinical development. Breakthrough Therapy designation provides all the features of Fast Track designation in addition to intensive guidance on an efficient drug development program beginning as early as Phase 1 and FDA organizational commitment to expedited development, including involvement of senior managers and experienced review staff in a cross-disciplinary review, where appropriate.

Any product submitted to the FDA for approval, including a product with Fast Track or Breakthrough Therapy designation, may also be eligible for additional FDA programs intended to expedite the review and approval process, including Priority Review designation and Accelerated Approval. A product is eligible for Priority Review if it has the potential to provide a significant improvement in safety or effectiveness in the treatment, diagnosis or prevention of a serious disease or condition. Under Priority Review, the FDA must review an application within six months, compared to ten months for a standard review.

Additionally, products are eligible for Accelerated Approval if they can be shown to have an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or an effect on a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality which is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments.

Accelerated Approval is usually contingent on a sponsor's agreement to conduct additional post-approval studies to verify and describe the product's clinical benefit. The FDA may 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, unless otherwise informed by the FDA, the FDA currently requires, as a condition for Accelerated Approval, that all advertising and promotional materials that are intended for dissemination or publication within 120 days following marketing approval be submitted to the agency for review during the pre-approval review period, and that after 120 days following marketing approval, all advertising and promotional materials must be submitted at least 30 days prior to the intended time of initial dissemination or publication.

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 the time period for FDA review or approval may not be shortened. Furthermore, Fast Track designation, Breakthrough Therapy designation, Priority Review and Accelerated Approval do not change the scientific or medical standards for approval or the quality of evidence necessary to support approval, but may expedite the development or review process.

U.S. Post-Approval Requirements for Drugs

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, reporting of adverse experiences with the product, complying with promotion and advertising requirements, which include restrictions on promoting products for unapproved uses or patient populations (known as "off-label use") and limitations on industry-sponsored scientific and educational activities.

Although physicians may prescribe legally available products for off-label uses, manufacturers may not market or promote such uses. 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 including investigation by federal and state authorities. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use or first publication. Further, if there are any modifications to the drug, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new NDA or NDA supplement which may require the development of additional data or preclinical studies and clinical trials.

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-market testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

In addition, drug manufacturers and their subcontractors involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies and are subject to periodic, unannounced inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements, including cGMP. These impose certain procedural and documentation requirements upon us and our contract manufacturers. Failure to comply with statutory and regulatory requirements can subject a manufacturer to possible legal or regulatory action, such as warning letters, suspension of manufacturing, product seizures, injunctions, civil penalties or criminal prosecution. There is also a continuing, annual prescription drug product program user fee.

Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, requirements for post market studies or clinical trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS. 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;
- safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve applications or supplements to approved applications, or withdrawal of product approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties; and
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs; or mandated modification of promotional materials and labeling and issuance of corrective information.

Other Regulatory Matters

Manufacturing, sales, promotion and other activities of product candidates following product approval, where applicable, or commercialization are also subject to regulation by numerous regulatory authorities in the U.S. in

addition to the FDA. These authorities may include the Centers for Medicare & Medicaid Services, or CMS, other divisions of the Department of Health and Human Services, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments and governmental agencies.

Healthcare Reform

The containment of health care costs is a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. In March 2010, Congress passed the Affordable Care Act, or the ACA, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of health spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional policy reforms. The ACA, for example, contains provisions that subject products to potential competition by lower-cost products and may reduce the profitability of products through increased rebates for drugs reimbursed by Medicaid programs, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increase the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations; establish annual fees and taxes on manufacturers of certain branded prescription drugs; and create a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% (increased to 70% pursuant to the Bipartisan Budget Act of 2018, or BBA, effective as of 2019) 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.

Since its enactment, there have been judicial, administrative, executive and Congressional legislative challenges to certain aspects of the ACA. We expect that there will be additional challenges and amendments to the ACA in the future. For example, on June 17, 2021, the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. In addition, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 ("IRA") into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and creating a new manufacturer discount program. It is unclear how any additional challenges and healthcare reform measures of the Trump administration will impact the ACA and our business.

Other federal health reform measures have been proposed and adopted in the U.S. since the ACA was enacted:

- The Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, including the BBA, will remain in effect through 2031, unless additional Congressional action is taken. Under current legislation the actual reduction in Medicare payments will vary from 1% in 2022 to up to 4% in the final fiscal year of this sequester.

- The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

- The Middle Class Tax Relief and Job Creation Act of 2012 required that CMS reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years.

- In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting.

Further, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products which have resulted in several recent Congressional inquiries and proposed and enacted bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, the U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs, including price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs to limit the growth of government paid healthcare costs. For example, the U.S. government has passed legislation requiring pharmaceutical manufacturers to provide rebates and discounts to certain entities and governmental payors to participate in federal healthcare programs. In addition, the IRA, among other things, (i) directs the U.S. Department of Health and Human Services ("HHS") to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare, and subject drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the negotiated "maximum fair price" for such drugs and biologics under the law, and (ii) imposes rebates with respect to certain drugs and biologics covered under Medicare Part B or Medicare Part D to penalize price increases that outpace inflation. The IRA permits HHS to implement many of these provisions through guidance as opposed to regulation for the initial years. These provisions will progressively take effect beginning in fiscal year 2023, although they may be subject to legal challenges. It is currently unclear how the IRA will be implemented but is likely to have a significant impact on the pharmaceutical industry. Further, the Biden administration released an additional executive order on October 14, 2022 directing HHS to submit a report on how the Center for Medicare and Medicaid ("CMS") Innovation can be further leveraged to test new models for lowering drug costs for Medicare and Medicaid beneficiaries. It is unclear whether this executive order or similar policy initiatives will be implemented in the future. Individual states in the U.S. have also been increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

From time to time legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the sale, marketing, coverage and reimbursement of products regulated by CMS or other government agencies. In addition to new legislation, CMS regulations and policies are often revised or interpreted by the agency in ways significantly affecting our business and our products.

Other Healthcare Laws and Regulations

If we obtain regulatory approval of our products, then we may be subject to various federal and state laws targeting fraud and abuse in the healthcare industry. These laws may impact, among other things, our proposed sales and marketing strategies. In addition, we may be subject to patient privacy regulations by both the federal government and the states in which we conduct our business. These laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security and physician sunshine laws and regulations.

The federal Anti-Kickback Statute prohibits, among other things, any person from knowingly and willfully offering, soliciting, receiving or paying remuneration (a term interpreted broadly to include anything of value, including, for example, gifts, discounts and credits), directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, an item or reimbursable, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. Violations of the federal Anti-Kickback Statute can result in significant civil monetary and criminal penalties per kickback plus three times the amount of remuneration and a prison term per violation. Further, violation of the federal Anti-Kickback Statute can also form the basis for False Claims Act liability (discussed below). 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. In addition, many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare items or services reimbursed by not only government programs, but any source.

Additionally, the civil False Claims Act (the "FCA") prohibits knowingly presenting or causing the presentation of a false, fictitious or fraudulent claim for payment to the U.S. government. Actions under the FCA may be brought by the Attorney General or as a qui tam action by a private individual in the name of the government. Violations of the FCA can result in very significant monetary penalties for each false claim and treble the amount of the government's damages. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims. The federal government continues to use the FCA and the accompanying threat of significant liability in its investigations and

prosecutions of pharmaceutical and biotechnology companies throughout the U.S. Such investigations and prosecutions frequently involve, for example, the alleged promotion of products for unapproved uses and other sales and marketing practices. The government has obtained multi-million and multi-billion dollar settlements under the FCA in addition to individual criminal convictions under applicable criminal statutes. Given the significant size of actual and potential settlements, it is expected that the government will continue to devote substantial resources to investigating healthcare providers' and manufacturers' compliance with the FCA and other applicable fraud and abuse laws.

We may be subject to the federal Civil Monetary Penalties Law, which prohibits, among other things, the offering or transferring of remuneration to a Medicare or Medicaid beneficiary that the person knows or should know is likely to influence the beneficiary's selection of a particular supplier of Medicare or Medicaid payable items or services. Federal government price reporting laws require manufacturers to calculate and report complex pricing metrics to government programs.

The U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, includes a fraud and abuse provision referred to as the HIPAA All-Payor Fraud Law. This provision imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, 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.

We may also be subject to federal transparency laws, including the federal Physician Payment Sunshine Act, which was part of the ACA and requires manufacturers of certain drugs and biologics, among others, to track and disclose payments and other transfers of value they make to U.S. physicians (defined to include doctors, dentists, optometrists, podiatrists, and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals, as well as ownership and investment interests in the manufacturer held by such physicians and their immediate family members. This information is subsequently made publicly available in a searchable format on a CMS website. Failure to disclose required information may result in civil monetary penalties for all payments, transfers of value or ownership or investment interests that are not timely, accurately and completely reported in an annual submission. Certain states also mandate implementation of compliance programs, impose restrictions on drug manufacturer marketing practices and/or require the tracking and reporting of gifts, compensation and other remuneration to physicians and/or other healthcare providers.

As noted above, analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors including private insurers. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures. There are also state and local laws that require the registration of pharmaceutical sales representatives.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, imprisonment, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, any of which could adversely affect our ability to operate our business and our financial results. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Ensuring business arrangements comply with applicable healthcare

laws, as well as responding to possible investigations by government authorities, can be time- and resource consuming and can divert a company's attention from the business.

Data Privacy and Security

In the ordinary course of business we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal information such as clinical trial data and other health data. Accordingly, we may be subject to numerous data privacy and security obligations including federal, state, local, and foreign laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements and other obligations related to data privacy and security.

These frameworks are evolving and may impose potentially conflicting obligations. Such obligations may include, without limitation, the Federal Trade Commission Act, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 ("CPRA") (collectively, "CCPA"), the European Union's General Data Protection Regulation 2016/679 ("EU GDPR"), the EU GDPR as it forms part of United Kingdom law by virtue of section 3 of the European Union (Withdrawal) Act 2018 ("UK GDPR"), the ePrivacy Directive, and wiretapping laws. Further. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, imposes certain requirements relating to the privacy, security and transmission of protected health information.

Many states also have laws governing the privacy and security of health information and other personal information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts. For example, the CCPA applies to personal information of consumers, business representatives, and employees who are California residents, places increased privacy and security obligations on entities handling personal information of such California residents or households, requires covered companies to provide certain disclosures to such California residents about its data collection, use and sharing practices, and requires covered companies to provide such California residents with ways to opt-out of certain sales or transfers of personal information. In addition, the CPRA expanded the CCPA's requirements.

Additionally, European data privacy and security laws (including the EU GDPR and UK GDPR) impose significant and complex compliance obligations on companies that are subject to those laws, notably with respect to the processing of health-related data from the European Economic Area ("EEA") (comprised of the 27 EU Member States plus Iceland, Liechtenstein and Norway) or UK-based individuals.

Coverage and Reimbursement

Market acceptance and sales of approved products depends in part on the extent to which reimbursement for these drugs and related treatments will be available from third-party payors including government health administration authorities, managed care organizations and other private health insurers. Third-party payors decide which therapies they will pay for and establish reimbursement levels. Third-party payors often rely upon the Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. Additionally, a third-party payor's decision to provide coverage for a therapy does not imply that an adequate reimbursement rate will be approved. Even if favorable coverage and reimbursement status is attained for any product candidate, less favorable coverage policies and reimbursement rates may be implemented in the future. Patients are unlikely to use our drugs unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our drugs. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. If coverage and adequate reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our current and any future product candidates that we develop.

Government Regulation of Drugs Outside of the United States

To market any product outside of the U.S., we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, manufacturing, commercial sales and distribution of our products. To obtain a Marketing Authorization, or MA, for a product in the EEA, for example, an applicant must be established within the EEA. The applicant must submit a Marketing Authorization Application, or MAA, either under a centralized procedure administered by the European Medicines Agency (the "EMA") or one of the procedures administered by competent

authorities in the EEA countries (decentralized procedure, national procedure or mutual recognition procedure). The centralized procedure provides for the grant of a single MA by the European Commission that is valid for all EEA countries. Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for (i) medicinal products derived from biotechnological processes, (ii) products designated as orphan medicinal products, (iii) advanced therapy medicinal products, or ATMPs and (iv) products with a new active substance indicated for the treatment of HIV/AIDS, cancer, neurodegenerative diseases, 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 patients, authorization through the centralized procedure is optional on related approval.

Under the centralized procedure, the EMA's Committee for Medicinal Products for Human Use, or CHMP, conducts the initial assessment of a product. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing MA. Under the centralized procedure in the EEA, 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. Accelerated assessment may be granted by the CHMP in exceptional cases, when a medicinal product targeting an unmet medical need is expected to be of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. If the CHMP accepts a request for accelerated assessment, the time limit of 210 days will be reduced to 150 days (excluding clock stops). The CHMP can, however, revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment.

Unlike the centralized authorization procedure, the decentralized MA procedure requires a separate application to and leads to separate approval by, the competent authorities of each EEA country 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 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 EEA countries 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 Heads of Medicines Agencies' Coordination Group for Mutual Recognition and Decentralised Procedures – Human, or CMDh, for review. The subsequent decision of the European Commission is binding on all EEA countries.

The mutual recognition procedure allows companies that have a medicinal product already authorized in one EEA country to apply for this authorization to be recognized by the competent authorities in other EEA countries. Like the decentralized procedure, the mutual recognition procedure is based on the acceptance by the competent authorities of the EEA countries of the MA of a medicinal product by the competent authorities of other EEA countries. The holder of a national MA may submit an application to the competent authority of an EEA country requesting that this authority recognize the MA delivered by the competent authority of another EEA country.

An MA has, in principle, an initial validity of five years. The MA may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the EEA country in which the original MA was granted. To support the application, the MA holder must provide the EMA or the competent authority with a consolidated version of the eCTD (Common Technical Document) providing up-to-date data concerning the quality, safety and efficacy of the product, including all variations introduced since the MA was granted, at least nine months before the MA ceases to be valid. The European Commission or the competent authorities of the EEA countries may decide on justified grounds relating to pharmacovigilance to proceed with one further five year renewal period for the MA. Once subsequently definitively renewed, the MA 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 (for a centralized MA) or on the market of the authorizing EEA country within three years after authorization ceases to be valid (the so-called sunset clause).

Innovative products that target an unmet medical need and are expected to be of major public health interest may be eligible for a number of expedited development and review programs, such as the Priority Medicines, or PRIME, scheme, which provides incentives similar to the breakthrough therapy designation in the U.S. PRIME is a voluntary scheme aimed at enhancing the EMA's support for the development of medicinal products that target unmet medical needs. 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 medicinal product 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. 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 potentially accelerated MAA assessment once a dossier has been submitted.

In the EEA, a "conditional" MA may be granted in cases where all the required safety and efficacy data are not yet available. The European Commission may grant a conditional MA for a medicinal product if it is demonstrated that all of the following criteria are met: (i) the benefit-risk balance of the medicinal product is positive; (ii) it is likely that the applicant will be able to provide comprehensive data post-authorization; (iii) the medicinal product fulfils an unmet medical need; and (iv) the benefit of the immediate availability to patients of the medicinal product is greater than the risk inherent in the fact that additional data are still required. The conditional MA is subject to conditions to be fulfilled for generating the missing data or ensuring increased safety measures. It is valid for one year and must be renewed annually until all related conditions have been fulfilled. Once any pending studies are provided, the conditional MA can be converted into a traditional MA. However, if the conditions are not fulfilled within the timeframe set by the EMA and approved by the European Commission, the MA will cease to be renewed.

An MA may also be granted "under exceptional circumstances" where the applicant can show that it is unable to provide comprehensive data on efficacy and safety under normal conditions of use even after the product has been authorized and subject to specific procedures being introduced. These circumstances may arise in particular when the intended indications are very rare and, in the state of scientific knowledge at that time, it is not possible to provide comprehensive information, or when generating data may be contrary to generally accepted ethical principles. Like a conditional MA, an MA granted "under exceptional circumstances" is reserved to medicinal products intended to be authorized for treatment of rare diseases or unmet medical needs for which the applicant does not hold a complete data set that is required for the grant of a standard MA. However, unlike the conditional MA, an applicant for authorization in exceptional circumstances is not subsequently required to provide the missing data. Although the MA "under exceptional circumstances" is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually and the MA will be withdrawn if the risk-benefit ratio is no longer favorable.

Upon grant of a MA in the EEA, innovative medicinal products generally benefit from eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents generic or biosimilar applicants from referencing the innovator's pre-clinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar marketing authorization during a period of eight years from the date on which the reference product was first authorized in the EEA. 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. The overall ten-year period will be extended to a maximum of eleven 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 authorization, is held to bring a significant clinical benefit in comparison with existing therapies.

In the EEA, there is a special regime for biosimilars, or biological medicinal products that are similar to a reference medicinal product but that do not meet the definition of a generic medicinal product. For such products, the results of appropriate preclinical or clinical trials must be provided in support of an application for marketing authorization. Guidelines from the EMA detail the type of quantity of supplementary data to be provided for different types of biological product.

EU Post-Approval Requirements

Where an MA is granted in relation to a medicinal product in the EEA, the holder of the MA is required to comply with a range of regulatory requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. Similar to the United States, both MA holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA, the European Commission and/or the competent regulatory authorities of the individual EEA countries. The holder of an MA must establish and maintain a pharmacovigilance system and appoint an individual qualified person for pharmacovigilance who is responsible for oversight of that

system. Key obligations include expedited reporting of suspected serious adverse reactions and submission of periodic safety update reports, or PSURs.

All new MAAs must include a risk management plan, or RMP, describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. The regulatory authorities may also impose specific obligations as a condition of the MA. Such risk-minimization measures or post-authorization obligations may include additional safety monitoring, more frequent submission of PSURs, or the conduct of additional clinical trials or post-authorization safety studies.

In the EEA, the advertising and promotion of medicinal products are subject to both EU and EEA countries' laws governing promotion of medicinal products, interactions with physicians and other healthcare professionals, misleading and comparative advertising and unfair commercial practices. Although general requirements for advertising and promotion of medicinal products are established under EU legislation, the details are governed by regulations in individual EEA countries and can differ from one country to another. For example, applicable laws require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics, or SmPC, as approved by the competent authorities in connection with an MA. The SmPC is the document that provides information to physicians concerning the safe and effective use of the product. Promotional activity that does not comply with the SmPC is considered off-label and is prohibited in the EEA. Direct-to-consumer advertising of prescription medicinal products is also prohibited in the EEA.

Orphan designation in the EU

The criteria for designating an "orphan medicinal product" in the EEA is similar in principle to that in the U.S. In the EEA, a medicinal product may be designated as orphan if (i) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (ii) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (iii) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. During this ten-year orphan market exclusivity period, no marketing authorization application shall be accepted, and no marketing authorization shall be granted for a similar medicinal product for the same indication. An orphan product can also benefit from an additional two years of market exclusivity in the EU for pediatric studies. The ten-year market exclusivity period may 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, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if (i) the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior; (ii) the applicant consents to a second orphan medicinal product application; or (iii) the applicant cannot supply enough orphan medicinal product.

Similar to the U.S., the various phases of non-clinical and clinical research in the EU are subject to significant regulatory controls.

Conduct of clinical trials in the EU

In the EU, clinical trials are governed by the Clinical Trials Regulation (EU) No 536/2014, or CTR, which entered into application on January 31, 2022, repealing and replacing the former Clinical Trials Directive 2001/20, or CTD, and related national implementing legislation of EU Member States.

The CTR is intended to harmonize and streamline clinical trial authorizations, simplify adverse-event reporting procedures, improve the supervision of clinical trials and increase their transparency. Specifically, the Regulation, which is directly applicable in all EU Member States, introduces a streamlined application procedure through a single-entry point, the "EU portal", the Clinical Trials Information System, or CTIS; a single set of documents to be prepared and submitted for the application; as well as simplified reporting procedures for clinical trial sponsors. A harmonized procedure for the assessment of applications for clinical trials has been introduced and is divided into two parts. Part I assessment is led by the competent authorities of a reference Member State selected by the trial sponsor and relates to clinical trial aspects that are considered to be scientifically harmonized across EU

Member States. This assessment is then submitted to the competent authorities of all concerned Member States in which the trial is to be conducted for their review. Part II is assessed separately by the competent authorities and Ethics Committees in each concerned EU Member State. Individual EU Member States retain the power to authorize the conduct of clinical trials on their territory.

Should we utilize third-party distributors, compliance with such foreign governmental regulations would generally be the responsibility of such distributors, who may be independent contractors over whom we have limited control.

The position in the United Kingdom

Following the result of a referendum in 2016, the United Kingdom (UK) left the European Union on January 31, 2020, commonly referred to as Brexit. The UK and the European Union have signed an EU-UK Trade and Cooperation Agreement, or TCA, which became provisionally applicable on January 1, 2021, and entered into force on May 1, 2021. The Annex provides a framework for the recognition of Good Manufacturing Practice, or GMP, inspections and for the exchange and acceptance of official GMP documents. The regime does not, however, extend to procedures such as batch release certification.

As part of the TCA, the European Union and the UK will recognize Good Manufacturing Practice inspections carried out by the other party and the acceptance of official GMP documents issued by the other party. The TCA also encourages, although it does not oblige, the parties to consult one another on proposals to introduce significant changes to technical regulations or inspection procedures. Among the areas of absence of mutual recognition are batch testing and batch release. The UK has unilaterally agreed to accept European Union batch testing and batch release. However, the European Union continues to apply European Union laws that require batch testing and batch release to take place in the European Union territory. This means that medicinal products that are tested and released in the UK must be retested and re-released when entering the European Union market for commercial use.

In regard to marketing authorizations, Great Britain has a separate regulatory submission process, approval process and a national marketing authorization. Northern Ireland will, however, continue to be covered by the marketing authorizations granted by the European Commission. Since January 1, 2021, an applicant for a centralized procedure marketing authorization can no longer be established in the UK. Since this date, companies established in the UK cannot use the centralized procedure and instead must follow one of the UK national authorization procedures to obtain an MA to market products in the UK. The MHRA has been updating various aspects of the regulatory regime for medicinal products in the UK. These include introducing the Innovative Licensing and Access Procedure to accelerate the time to market and facilitate patient access for innovative medicinal products, updates to the UK national approval procedure, introduction of a 150-day objective for assessing applications for marketing authorizations in the UK, Great Britain and Northern Ireland and a rolling review process for marketing authorization applications (rather than a consolidated full dossier submission).

Orphan designation in Great Britain following Brexit is, unlike in the EU, not available pre-marketing authorization. Applications for orphan designation are made at the same time as an application for a marketing authorization. The criteria to be granted as an orphan medicinal product designation is essentially identical to those in the EU but is based on the prevalence of the condition in Great Britain.

The UK regulatory framework in relation to clinical trials is derived from existing EU legislation (as implemented into UK law, through secondary legislation). However, it is currently unclear to what extent the UK will seek to align its regulations with the EU following entry into application of the Clinical Trials Regulation on January 31, 2022.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of FDA approval of our future product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit restoration of the patent term of up to five years as compensation for patent term lost during the FDA regulatory review process. Patent-term restoration, however, cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. The patent-term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA.

Marketing exclusivity provisions under the FD&C Act also can delay the submission or the approval of certain applications. The FD&C Act provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity 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 abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company 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, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FD&C Act also provides three years of marketing 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 covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. 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.

Employees and Human Capital Resources

As of December 31, 2024, we had twenty-three full-time employees and two part-time employees. Of these twenty-five employees, seventeen were engaged in research and development activities and eight were engaged in finance, business development, administrative support and general management. We have a branch office in Barcelona, Spain. Our employees in Spain are subject to a national collective labor agreement, the "Convenio General de la Industria Quimica". National agreements are negotiated collectively between the national associations of companies within a given industry and the respective national unions. We consider our relationship with our employees to be good and have not experienced any work stoppages. Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. We maintain our equity incentive plan in order to attract, retain and incentivize our workforce through the granting of stock-based compensation. We also provide cash bonus awards based on Company progress toward key annual goals and employee performance.

Available Information

We maintain an internet website at www.gaintherapeutics.com and make available free of charge through our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Sections 13(a) and 15(d) of the Exchange Act of 1934, or the Exchange Act. We make these reports available through our website as soon as reasonably practicable after we electronically file such reports with, or furnish such reports to, the Securities and Exchange Commission, or the SEC. You can review our electronically filed reports and other information that we file with the SEC on the SEC's web site at http://www.sec.gov. In addition, we regularly use our website to post information regarding our business, product

development programs and governance, and we encourage investors to use our website, specifically the section titled "Investor Relations" as a source of information about us. The information on our website is not incorporated by reference into this Annual Report and should not be considered to be part of this Annual Report. Our website address is included in this Annual Report as an inactive technical reference only.

ITEM 1A. RISK FACTORS

Investing in our securities involves a high degree of risk. You should carefully consider the following risks and other information included or incorporated by reference in this Annual Report in evaluating our company and our common stock. Any of the following risks could materially and adversely affect our results of operations, our financial condition and the market price of our common stock. Although the risk factors are grouped by general category, many of the risks described in a given category relate to multiple categories. The risks described below are not the only ones that we face. Additional risks not presently known to us or that we currently deem immaterial may also affect our business, operating results, prospects or financial condition. See "Cautionary Statement Regarding Forward-Looking Statements" in this Annual Report. If any of these risks actually materialize, our business, prospects, financial condition and results of operations could be seriously harmed. This could cause the trading price of our common stock to decline, resulting in a loss of all or part of your investment.

Risk Factor Summary

We are providing the following summary of the risk factors contained in this Annual Report to enhance the readability and accessibility of our risk factor disclosures. We encourage you to carefully review the full risk factors contained in this Annual Report in their entirety for additional information regarding the material factors that make an investment in our securities speculative or risky. These risks and uncertainties include, but are not limited to, the following:

- there is substantial doubt about our ability to continue as a going concern. We have a history of operating losses and expect to incur losses for the foreseeable future. We may never generate revenues or, if we are able to generate revenues, achieve profitability;
- we will need to raise additional capital and such financing may not be available to us in the necessary time frame, in amounts that we require, on terms that are acceptable to us, or at all;
- our ability to maintain compliance with the continued listing requirement of the Nasdaq Stock Market LLC ("Nasdaq");
- we have a limited operating history, and we expect a number of factors to cause our operating results to fluctuate on a quarterly and annual basis which may make it difficult to predict future performance;
- if preclinical studies or clinical trials for our product candidates cannot be initiated or completed or if they are delayed or unsuccessful, we will be unable to meet our future development and commercialization goals;
- some of the disorders we seek to treat have low prevalence and it may be difficult to identify patients with these disorders, which may lead to delays in enrollment for our trials or slower commercial revenue if approved, and we may also face enrollment challenges as a result of other factors;
- our product candidates are novel and still in development. If we are unable to successfully develop, receive regulatory approval for and commercialize our current or future product candidates, our business will be harmed;
- we have started testing one of our product candidates in clinical trials. Success in preclinical studies or clinical trials may not be indicative of results obtained in later clinical trials;
- clinical trials required for our product candidates are expensive and time-consuming, and their outcomes are uncertain;
- we will need to raise additional capital, which may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. Additional capital may not be available on favorable terms or at all which may force us to delay, reduce the scope of or eliminate our research and development programs, reduce our commercialization efforts or curtail our operations;
- we are subject to extensive and costly government regulations which are subject to change;

- even if we obtain regulatory approval to market our product candidates, our product candidates may not be accepted by the market;
- we rely on a license to use the technology that is material to our business and if the agreement underlying the license were to be terminated or if other rights that may be necessary for commercializing our intended products cannot be obtained, it would halt our ability to market our products and technology, as well as have an immediate material adverse effect on our business, operating results and financial condition;
- we are subject to stringent and evolving U.S. and foreign laws, regulations, rules, contractual obligations, policies and other obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions, litigation, (including class actions) and mass arbitration demands, fines and penalties, disruptions of our business operations, reputational harm, loss of revenue or profits and other adverse business consequences; and
- global and macroeconomic conditions, including worldwide economic, political and social instability could adversely affect our revenue, financial condition, or results of operations.

Risks Related to Our Business

There is substantial doubt about our ability to continue as a going concern. We have a history of operating losses and expect to incur losses for the foreseeable future. We may never generate revenues or, if we are able to generate revenues, achieve profitability.

We are focused on product development, and we have not generated any significant revenues to date. We have incurred losses in each year of our operations and we expect to continue to incur operating losses for the foreseeable future. Since our inception, we have incurred operating losses which have adversely affected, and are likely to continue to adversely affect, our working capital, total assets and shareholders' equity. In addition, the impact of these events and conditions on our liquidity raise substantial doubt about our ability to continue as a going concern.

We will need to raise additional capital and plan to raise additional capital primarily through public and/or private equity financings and/or convertible debt financings. However, financing may not be available to us in the necessary time frame, in amounts that we require, on terms that are acceptable to us or at all. If we are unable to raise the necessary funds when needed, it may materially and adversely impact our ability to execute on our operating plans. If we become unable to continue as a going concern, we may have to dispose of assets and might realize significantly less than the values at which they are carried on our consolidated financial statements. These actions may cause our stockholders to lose all or part of their investment in our common stock.

We and our prospects should be examined in light of the risks and difficulties frequently encountered by new and early-stage companies in new and rapidly evolving markets. These risks include, among other things, the speed at which we can scale up operations, our complete dependence upon development of our product candidates that currently have no market acceptance, our ability to establish and expand our brand name, our development of and reliance on strategic and customer relationships and our ability to minimize fraud and other security risks.

The process of developing our product candidates requires significant time, effort and expenses in preclinical, clinical and regulatory development. In addition, commercialization of our product candidates will require that we obtain necessary regulatory approvals and establish sales, marketing and manufacturing capabilities either through internal hiring or through contractual relationships with others. We expect to incur substantial additional operating expenses over the next several years as our research, development, preclinical studies and clinical trial activities increase. Product candidates in later stages of clinical development generally incur higher development costs than those in earlier stages of clinical development primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect that our research and development expenses will continue to increase in the foreseeable future as we (i) increase personnel costs, including stock-based compensation, (ii) continue preclinical development of our lead compounds, (iii) initiate clinical trials for certain product candidates, (iv) continue to discover and develop additional product candidates, and (v) pursue later stages of clinical development of product candidates.

The amount of future losses and when, if ever, we will achieve profitability are uncertain. We have no products that have generated any commercial revenue, do not expect to generate revenues from the commercial sale of products in the foreseeable future and might never generate revenues from the sale of products. Our ability to generate revenue and achieve profitability will depend on, among other things, successful completion of preclinical

development and testing and clinical trials of our product candidates; obtaining necessary regulatory approvals from the FDA and comparable foreign regulatory authorities; establishing manufacturing, sales and marketing arrangements with third parties; successfully commercializing our products; establishing a favorable competitive position; and raising sufficient funds to finance our activities. Many of these factors will depend on circumstances beyond our control. We might not succeed at any of these undertakings. If we are unsuccessful at some or all of these undertakings, our business, prospects and results of operations may be materially adversely affected.

We have a limited operating history and we expect a number of factors to cause our operating results to fluctuate on a quarterly and annual basis which may make it difficult to predict our future performance.

We are an early clinical stage biopharmaceutical company with a limited operating history. Our operations to date have been primarily limited to organizing and staffing our company, expanding its operations, performing research, acquiring, developing and securing our in-licensed technology and preclinical development of our product candidates. We have commenced our first Phase 1 clinical trial but not yet successfully completed any clinical trials, manufactured our products candidates at commercial scale or conducted sales and marketing activities that will be necessary to successfully commercialize our product candidates, if approved. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history or commercialized products. Our financial condition has varied significantly in the past and will continue to fluctuate from quarter-to-quarter or year-to-year due to a variety of factors, many of which are beyond our control. Factors relating to our business that may contribute to these fluctuations, among other factors described elsewhere in this Annual Report, include:

- our ability to obtain additional funding to develop our product candidates, the extent to which we are able to obtain such funding on favorable terms, and changes to our operations or strategy that may be necessitated due to the need for additional funding;
- our ability to conduct and complete preclinical studies, including GLP-compliant and IND-enabling preclinical studies;
- delays in the commencement, enrollment and timing of clinical trials;
- the success of our preclinical studies and clinical trials through all phases of development;
- any delays in regulatory review and approval of product candidates in clinical development;
- our ability to obtain and maintain regulatory approval for our product candidates in the U.S. and foreign jurisdictions;
- our ability to successfully commercialize product candidates for which we obtain regulatory approval within expected timelines or at all;
- potential toxicity and/or side effects of our product candidates that could delay or prevent commercialization, limit the indications for any approved drug, require the establishment of risk evaluation and mitigation strategies ("REMS"), or comparable foreign strategies or cause an approved drug to be taken off the market;
- our ability to establish or maintain collaborations, licensing or other arrangements;
- market acceptance of our product candidates;
- competition from existing products, new products or new therapeutic approaches that may emerge;
- the ability of patients or healthcare providers to obtain coverage of or sufficient reimbursement for our products;
- our ability to leverage our in-licensed technology platform to discover and develop additional product candidates;

- our ability and our licensors' abilities to successfully obtain, maintain, defend and enforce intellectual property rights important to our business;

- the impact of political instability, natural disasters, events of terrorism and wars, including Russia's invasion of Ukraine and the conflict between Hamas and Israel;

- the impact of other global and macroeconomic conditions, including heightened inflation and high interest rates, liquidity concerns at and failures of banks and other financial institutions, supply chain disruptions, fluctuating exchange rates and increases in commodity, energy and fuel prices; and

- potential product liability claims.

Accordingly, the results of any quarterly or annual periods should not be relied upon as indications of future operating performance.

Risks Related to Product Development, Regulatory Approval, Manufacturing and Commercialization

We are subject to extensive and costly government regulation, which are subject to change.

Product candidates employing our technology are subject to extensive and rigorous domestic government regulation including regulation by the FDA, the Centers for Medicare and Medicaid Services ("CMS"), other divisions of the United States Department of Health and Human Services ("HHS"), the United States Department of Justice ("DOJ"), state and local governments and their respective foreign equivalents. The FDA and comparable foreign regulatory authorities regulate the research, development, preclinical studies and clinical trials, manufacture, safety, effectiveness, recordkeeping, reporting, labeling, storage, approval, advertising, promotion, sale, distribution, import and export of biopharmaceutical products. If products employing our technologies are marketed abroad, they will also be subject to extensive regulation by foreign governments, whether they have obtained the FDA's or comparable foreign regulatory authorities' approval for a given product and its uses. Such foreign regulation may be equally or more demanding than corresponding U.S. regulation.

Government regulation substantially increases the cost and risk of researching, developing, manufacturing and selling our products. The regulatory review and approval process, which includes preclinical studies and clinical trials of each product candidate, is lengthy, expensive, and uncertain. We or our collaborators must obtain and maintain regulatory authorization to conduct clinical trials as well as approval for each product we intend to market and the manufacturing facilities used for the products must be inspected and meet legal requirements. Securing regulatory approval requires the submission of extensive preclinical and clinical data and other supporting information for each proposed therapeutic indication to establish the product's safety and efficacy, and in the case of biologics also potency and purity, for each intended use. The development and approval process takes many years, requires substantial resources and may never lead to the approval of a product.

Even if we can obtain regulatory approval for a particular product, the approval may limit the indicated medical uses for the product, may otherwise limit our ability to promote, sell, and distribute the product, may require that we conduct costly post-marketing surveillance, and/or may require that we conduct ongoing post marketing studies. Material changes to an approved product, such as, for example, manufacturing changes or revised labeling, may require further regulatory review and approval. Once obtained, any approvals may be withdrawn, suspended or varied. For example, if there is a later discovery of previously unknown problems with the product, such as a previously unknown safety issue.

If we, our collaborators or our manufacturers fail to comply with applicable regulatory requirements at any stage during the regulatory process, such noncompliance could result in, among other things, delays in the approval of applications or supplements to approved applications; refusal of a regulatory authority, including the FDA, to review pending market approval applications or supplements to approved applications; warning letters; fines; import and/or export restrictions; product recalls or seizures; injunctions; total or partial suspension of production, distribution, manufacturing or clinical trials; civil penalties; withdrawals, suspension or variation of previously approved marketing applications or licenses; recommendations by the FDA or comparable foreign other regulatory authorities against governmental contracts; and/or criminal prosecutions.

Moreover, the development of our product candidates may be delayed by other events beyond our control. For example, action by the Trump administration to limit federal agency budgets or personnel, may result in reductions to the FDA's budget, employees, and operations, which may lead to slower response times and longer review periods, potentially affecting our ability to progress development of our product candidates or obtain regulatory approval for our product candidates.

Our long-term success depends heavily on our ability to fund and complete research and development activities and obtain regulatory approval for our product candidates.

Only a small minority of all research and development programs ultimately result in commercially successful drugs. Clinical failure can occur at any stage of clinical development. Clinical and preclinical trials may produce negative or inconclusive results and we may decide, or regulators may require us, to conduct additional clinical or preclinical trials. In addition, data obtained from trials are susceptible to varying interpretations and regulators may not interpret our data as favorably as we do. This may delay, limit or prevent regulatory approval. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a drug candidate. As part of development, we also must show that we can formulate and manufacture our product candidates in compliance with regulatory requirements**.**

We will need substantial additional financing to complete the development of our drug candidates and comply with the regulatory requirements governing this process. Further, even if we complete the development of our drug candidates and gain marketing approvals from the FDA and comparable foreign regulatory authorities in a timely manner, we cannot be sure that such drug candidates will be commercially successful in the pharmaceutical market. If the results of clinical trials, the anticipated or actual timing of marketing approvals or the market acceptance of any of our drug candidates, if approved, do not meet the expectations of investors or public market analysts, our business will be in jeopardy and the market price of our common stock would likely decline.

If preclinical studies or clinical trials for our product candidates cannot be initiated or completed or if they are delayed or unsuccessful, we will be unable to meet our future development and commercialization goals.

Delays in preclinical studies and clinical trials could significantly increase our product development costs and delay product commercialization. In addition, many of the factors that may cause or lead to a delay in the clinical trials may also ultimately lead to denial of regulatory approval of a product candidate.

The commencement of clinical trials can be delayed for a variety of reasons, including delays in:

- demonstrating sufficient safety and efficacy to obtain regulatory approval to commence a clinical trial;
- reaching agreement on acceptable terms with prospective contract research organizations ("CROs") and study sites;
- developing a stable formulation of a product candidate;
- manufacturing sufficient quantities of a product candidate; and
- obtaining institutional review board ("IRB") approval or ethic committee opinions to conduct a clinical trial at a prospective site.

Once a clinical trial has begun, it may be delayed, suspended or terminated by us or the FDA or other comparable foreign regulatory authorities due to a number of factors including:

- ongoing discussions with the FDA or other comparable foreign regulatory authorities regarding the scope or design of our clinical trials;
- failure to conduct clinical trials in accordance with regulatory requirements;
- lower than anticipated recruitment or retention rate of patients in clinical trials;

- inspection of the clinical trial operations or study sites by the FDA or other comparable foreign regulatory authorities resulting in the imposition of a clinical hold;
- lack of adequate funding to continue clinical trials;
- negative results of clinical trials;
- investigational drug product out-of-specification; or
- nonclinical or clinical safety observations, including adverse events and SAEs.

We rely and expect to continue to rely on third parties including CROs and outside consultants to conduct, supervise or monitor some or all aspects of preclinical studies and clinical trials involving our product candidates. We have less control over the timing and other aspects of these preclinical studies and clinical trials than if we performed the monitoring and supervision entirely on our own. Third parties may not perform their responsibilities for our preclinical studies and clinical trials on our anticipated schedule or, for clinical trials, consistent with a clinical trial protocol.

If clinical trials are unsuccessful and we are not able to obtain regulatory approvals for our product candidates under development, we will not be able to commercialize these products and therefore may not be able to generate sufficient revenues to support our business.

Some of the disorders we seek to treat have low prevalence and it may be difficult to identify patients with these disorders, which may lead to delays in enrollment for our trials or slower commercial revenue if approved, and we may also face enrollment challenges as a result of other factors.

As our portfolio of drug candidates moves from preclinical testing to clinical testing and then through progressively larger and more complex clinical trials, we will need to enroll an increasing number of patients with the appropriate characteristics. At times we have experienced difficulty enrolling patients and we may experience more difficulty as the scale of our clinical testing program increases. The factors that affect our ability to enroll patients are largely uncontrollable and principally include the following:

- the size of the patient population;
- the severity of the disease under investigation;
- the nature of the clinical test and design of the study protocol;
- the eligibility criteria for the trial;
- the perceived risks, benefits and convenience of administration of the product candidate being studied;
- our efforts to facilitate timely enrollment in clinical trials;
- the availability of other clinical trials being conducted for the same indication;
- the patient referral practices of physicians; and
- the proximity and availability of clinical trial sites to prospective patients.

Our inability to enroll a sufficient number of patients with these diseases in our future clinical trials would result in significant delays and could prevent us from initiating or cause us to abandon clinical trials for one or more indications altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates which would cause the value of our company to decline and limit our ability to obtain additional financing.

Additionally, the reported number of people in some of the indications we aim to treat, as well as the people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. The total addressable market opportunity for our product candidates will ultimately depend upon, among other things, the final approved product labeling for each of our product candidates, if our product candidates are approved for sale in our target indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients globally may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our products or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

Our product candidates are novel and still in development. If we are unable to successfully develop, receive regulatory approval for and commercialize our current or future product candidates, our business will be harmed.

Because the Magellan™ platform remains untested and our product candidates are in early stages of development, they will require extensive preclinical and clinical testing. Our product candidates will require significant additional development, preclinical and IND-enabling studies and clinical trials, regulatory clearances and additional investment by us or our collaborators before they can be commercialized. Our drug development methods may not lead to commercially viable drugs for any of several reasons. For example, we may fail to identify appropriate targets or compounds, our product candidates may fail to be safe and effective in clinical trials or we may have inadequate financial or other resources to pursue development efforts for our product candidates. Also, third parties we rely on for preclinical development, such as the providers of supercomputer time needed for our Magellan™ platform and collaborators that provide us with materials and resources may fail to fulfill their obligations to us in a timely manner or at all and the development of our product candidates could be significantly delayed as a result. In addition, we are still developing proof of concept for our product candidates in animals and positive data from animal models may not be predictive of positive human results. Patients may have side effects that were not observed in animals.

Further, we and our product candidates are subject to extensive regulation by the FDA and comparable regulatory authorities in other countries governing, among other things, research, testing, clinical trials, manufacturing, labeling, promotion, selling, adverse event reporting and recordkeeping. Obtaining FDA and comparable foreign regulatory authority approval is a lengthy, expensive and uncertain process. If required regulatory registrations or approvals are delayed, denied, withdrawn, suspended or varied or if the regulatory authorities question the efficacy of our new small molecules as a treatment, such events are likely to have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.

Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials.

We will be required to demonstrate through adequate and well-controlled clinical trials that our product candidates are safe and effective, with a favorable benefit-risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. Trial designs and results from early-phase trials are not necessarily predictive of future clinical trial designs or results and initial positive results we may observe may not be confirmed in later-phase clinical trials. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development even if they successfully advance through initial clinical trials. We may not be able to demonstrate the safety and efficacy of our STAR molecules in our clinical trials. Even if our clinical trials demonstrate acceptable safety and efficacy of STAR molecules for a targeted disease, the labeling we obtain through negotiations with the FDA or comparable foreign regulatory authorities may not include data on secondary endpoints and may not provide us with a competitive advantage over other products approved for the same or similar indications. Many companies in the biotechnology industry have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and there is a high failure rate for product candidates proceeding through clinical trials. We may face similar setbacks or failures. Different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or comparable foreign regulatory authorities. Preclinical and clinical data can be interpreted in different ways. Accordingly, the FDA or comparable foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent regulatory approval. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, then the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld, varied or withdrawn. Regulatory delays

or rejections may also be encountered as a result of many other factors including changes in regulatory policy during the period of product development.

The approach we are taking to discover and develop our product candidates is novel and may never lead to marketable products.

We have concentrated our efforts and research and development activities on our novel small molecules for potential treatment of rare and genetic diseases and on more prevalent neurodegenerative diseases caused by protein misfolding and Magellan™, our target identification platform. Our future success depends on the successful development of such product candidates, including our ability to successfully complete IND-enabling and GLP-compliant preclinical studies, and the effectiveness of our platform. The scientific discoveries that form the basis for our efforts to discover and develop new drugs are relatively new. The scientific evidence to support the feasibility of developing drugs based on these discoveries is both preliminary and limited. Skepticism as to the feasibility of developing small molecules of this type that can cross the blood-brain barrier generally has been, and may continue to be, expressed in scientific literature. In addition, decisions by other companies with respect to their therapeutic development efforts may increase skepticism in the marketplace regarding the potential for potential therapeutics. There are currently no companies with approved disease modifying small molecule drugs for these indications that have the ability to cross the blood-brain barrier.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and human resources, we are currently focusing primarily on development of our Parkinson's program. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. 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 programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Clinical trials required for our product candidates are expensive and time-consuming, and their outcome is uncertain.

To obtain FDA or comparable foreign regulatory authority approval to market a new pharmaceutical product, we must demonstrate proof of safety and effectiveness in humans. To meet these requirements, we must conduct "adequate and well controlled" clinical trials. Conducting clinical trials is a lengthy, time-consuming and expensive process. The length of time may vary substantially according to the type, complexity, novelty and intended use of the product candidate, and often can be several years or more per study. Delays in clinical trials for our product candidates may cause us to incur additional operating expenses. The commencement and rate of completion of clinical trials may be delayed by many factors, including, for example: inability to manufacture sufficient quantities of stable and qualified materials under current good manufacturing practices ("cGMPs") for use in clinical trials; slower than expected rates of patient recruitment; failure to recruit a sufficient number of patients, which is a common issue in studies for rare disorders such as some of the indications we are currently pursuing; modification of clinical trial protocols; changes in regulatory requirements for clinical trials; the lack of effectiveness during clinical trials; the emergence of unforeseen safety issues; delays, suspension, or termination of the clinical trials due to the investigatory authority responsible for overseeing the trial at a particular trial site; and government or regulatory delays or "clinical holds" requiring suspension or termination of the studies.

Our clinical trials may be conducted in patients with neurodegenerative diseases, and in some cases, our product candidates are expected to be used in combination with approved therapies that themselves have significant adverse event profiles. During the course of treatment, these patients could suffer adverse medical events or die for reasons that may or may not be related to our product candidates. Any safety issues that arise with respect to our product candidates may delay or prevent clinical development.

The failure of clinical trials to demonstrate safety and effectiveness for the desired indications could harm the development of that product candidate and other product candidates that use a similar therapeutic approach. This failure could cause us to abandon a product candidate and could delay development of other product candidates. Any delay in or termination of our clinical trials would delay our ability to obtain regulatory approvals for and commercialization of our product candidates and generate product revenues. Any change in or termination of our clinical trials could materially harm our business, financial condition and results of operations.

We have limited experience as a company conducting clinical trials and may be unable to complete pivotal clinical trials for any product candidates we may develop.

Our success is dependent upon our ability to initiate and successfully complete clinical trials and obtain regulatory approval for and commercialization of our product candidates. We have not demonstrated an ability to perform the functions necessary for the approval or successful commercialization of any product candidate. The successful commercialization of any product candidate may require us to perform a variety of functions including:

- continuing to undertake preclinical development;
- obtaining approval to commence clinical trials;
- successfully planning and enrolling subjects in clinical trials;
- participating in regulatory approval processes;
- formulating and manufacturing products; and
- conducting sales and marketing activities.

We have limited experience designing, conducting and enrolling subjects in clinical trials. Until recently, our operations have been limited primarily to organizing and staffing our company, expanding its operations, performing research, acquiring, developing and securing our in-licensed technology and preclinical development of our product candidates. These operations provide a limited basis to assess our ability to develop and commercialize our product candidates.

Because of this lack of experience, any clinical trials we may conduct may not be completed on time, if at all. Large-scale trials require significant additional financial and management resources, monitoring and oversight and reliance on third-party clinical investigators, consultants or CROs. Relying on third-party clinical investigators, CROs and manufacturers, which are all also subject to governmental oversight and regulations, may also cause us to encounter delays that are outside of our control.

In addition, we may be unable to successfully and efficiently advance any candidates we select for clinical trials or execute and complete necessary GLP-compliant preclinical and IND-enabling studies in a way that leads to IND submission, successful development and ultimately commercial approval of any product candidate. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of any product candidates that we develop. Failure to commence or complete, or delays in, future planned clinical trials, could prevent us from or delay us in commercializing our product candidates.

We may conduct certain of our clinical trials for our product candidates outside of the U.S. which, among other risks, exposes us to the possibility that the FDA and other comparable foreign regulatory authorities may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business.

We have recently completed a first-in-human Phase 1 clinical trial in healthy volunteers for our Parkinson's disease program in Australia. During a pre-IND meeting with the FDA that took place in December 2024, we reviewed all of the clinical and preclinical data we have recorded for GT-02287 thus far, including the first-in-human Phase 1 study, and discussed our plans to submit an IND application for US expansion of clinical development for GT-02287 in support of eventual marketing approval in the U.S. Where data from foreign clinical trials are intended to serve as the basis for marketing approval in the U.S., the FDA will not approve the application on the basis of foreign data alone unless those data are applicable to the U.S. population and U.S. medical practice. Therefore, later stage clinical

trials designed to determine that GT-02287 is safe and effective for the purposes of FDA approval will be conducted in part in the U.S. The Phase 1 study was performed by clinical investigators of recognized competence; and the data are considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. For studies that are conducted only at sites outside of the U.S. and not subject to an IND, the FDA requires the clinical trial to have been conducted in accordance with GCPs and the FDA must be able to validate the data from the clinical trial through an on-site inspection if it deems such inspection necessary. For such studies not subject to an IND, the FDA generally does not provide advance comment on the clinical protocols for the studies, and therefore there is an additional potential risk that the FDA could determine that the study design or protocol for a non-U.S. clinical trial was inadequate, which could require us to conduct additional clinical trials. There can be no assurance the FDA will accept data from clinical trials conducted outside of the United States. If the FDA does not accept data from our clinical trials of our product candidates conducted outside of the United States, it would likely result in the need for additional clinical trials, which would be costly and time consuming and delay or permanently halt our development of our product candidates.

Conducting clinical trials outside the United States also exposes us to additional risks including risks associated with:

- additional foreign regulatory requirements;
- foreign exchange fluctuations;
- compliance with foreign manufacturing, customs, shipment and storage requirements;
- cultural differences in medical practice and clinical research; and
- diminished protection of intellectual property in some countries.

By extension, clinical trials that are predominantly conducted in the U.S. or primarily based on feedback from the FDA may not result in sufficiently diverse patient populations to warrant approval in other countries (for example, Japan) or those other comparable foreign regulatory authorities may have differences of opinion on appropriateness of trial design or differences in interpretation of some data. In those situations, approvals in other countries outside the U.S. may be delayed or never approved, which would materially detract from the commercial success of any impacted product candidates.

If we decide to pursue a Fast Track Designation or comparable foreign regulatory procedures for some of our product candidates, it may not lead to a faster development or regulatory review or approval process.

We may seek Fast Track Designation, or comparable foreign regulatory procedures, for one or more of our product candidates. If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the product sponsor may apply for FDA Fast Track Designation. The FDA has broad discretion whether to grant this designation, so even if we believe a particular product candidate is eligible for this designation, the FDA may decide not 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 withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program. The EMA has a similar program called PRIME.

If we decide to seek Orphan Drug Designation for some of our product candidates, we may be unsuccessful or may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for supplemental market exclusivity.

As part of our business strategy, we may seek Orphan Drug Designation for one or more of our product candidates which may be unsuccessful. Regulatory authorities in some jurisdictions, including the United States and European countries, 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 individuals in the U.S. or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the U.S., Orphan Drug Designation entitles a party to financial incentives such as a tax credit.

Opportunities for grant funding toward clinical trial costs may also be available for clinical trials of drugs for rare diseases, regardless of whether the drugs are designated for the orphan use. In addition, if a product that has Orphan Drug Designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity which means that the FDA may not approve any other applications to market the same product for the same indication for seven years, except in limited circumstances.

Even if we obtain Orphan Drug Designation for our product candidates in specific indications, we may not be the first to obtain marketing approval of these product candidates for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products. If a competitor with a product that is determined by the FDA to be the same as one of our product candidates obtains marketing approval before us for the same indication we are pursuing and obtains orphan drug exclusivity, our product candidate may not be approved until the period of exclusivity ends unless we are able to demonstrate that our product candidate is clinically superior. Even after obtaining approval, we may be limited in our ability to market our product. In addition, exclusive marketing rights in the U.S. may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost 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. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different principal molecular structural features can be approved for the same condition.

In the European Union (the "EU"), Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a medicinal product can be designated as an orphan drug by the European Commission if its sponsor can establish that: (i) the product is intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions; (ii) either (a) such conditions affect not more than 5 in 10,000 persons in the EU when the application is made, or (b) the product without the benefits derived from orphan status, would not generate sufficient return in the EU to justify the necessary investment in developing the medicinal product; and (iii) there exists no satisfactory authorized method of diagnosis, prevention, or treatment of the condition that has been authorized in the EU, or even if such method exists, the product will be of significant benefit to those affected by that condition.

Orphan medicinal product designation entitles an applicant to incentives such as fee reductions or fee waivers, protocol assistance, and access to the centralized marketing authorization procedure. Upon grant of a marketing authorization, orphan medicinal products are entitled to a ten-year period of market exclusivity for the approved therapeutic indication, which means that the EMA cannot accept another marketing authorization application or accept an application to extend for a similar product and the European Commission cannot grant a marketing authorization for the same indication for a period of ten years. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed pediatric investigation plan. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications. Orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The period of market exclusivity may, however, be reduced to six (6) years if, at the end of the fifth year, it is established that the product no longer meets the criteria on the basis of which it received orphan medicinal product destination, including where it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity or where the prevalence of the condition has increased above the threshold. Additionally, a marketing authorization may be granted to a similar medicinal product with the same orphan indication during the 10 year period if: (i) if the applicant consents to a second original orphan medicinal product application, (ii) if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities; or (iii) if the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A company may voluntarily remove a product from the register of orphan products.

Orphan Drug Designation in the U.S. or orphan medicinal product designation the EU neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process. While we may seek Orphan Drug Designation in the U.S. or orphan medicinal product designation the EU for our product candidates, we may never receive such designation.

We do not have, and may never obtain, the regulatory approvals we need to market our product candidates.

Following completion of clinical trials, the results are evaluated and, depending on the outcome, a New Drug Application ("NDA") is submitted to the FDA to obtain the FDA's approval of the product and authorization to commence commercial marketing. In responding to an NDA, the FDA may require additional testing or information, may require that the product labeling be modified, may impose post-approval study and other commitments or reporting requirements or other restrictions on product distribution, or may deny the application. The FDA has established performance goals for review of NDAs: six (6) months for priority applications and ten (10) months for standard applications. However, the FDA is not required to complete its review within these time periods. The timing of final review by the FDA and action varies greatly but can take years in some cases and may involve the input of an FDA advisory committee of outside experts. Product sales in the U.S. may commence only when an NDA is approved. Comparable procedures and limitations are applicable in the EU and in other jurisdictions.

To date, we have not applied for or received the regulatory approvals required for the commercial sale of any of our products in the United States or in any foreign jurisdiction. None of our product candidates have been determined to be safe and effective, and we have not submitted an NDA to the FDA or an equivalent application to any comparable foreign regulatory authorities for any of our product candidates.

It is possible that none of our product candidates will be approved for marketing. Failure to obtain regulatory approvals or delays in obtaining regulatory approvals may adversely affect the successful commercialization of any drugs or biologics that we or our partners develop, may impose additional costs on us or our collaborators, may diminish any competitive advantages that we or our partners may attain and/or may adversely affect our receipt of revenues or royalties.

Our product candidates may cause serious adverse events ("SAEs") or undesirable side effects which may delay or prevent marketing approval, or, if approval is received, require them to be taken off the market, require them to include safety warnings or otherwise limit their sales.

SAEs or undesirable side effects from our product candidates could arise either during development or, if approved, after the approved product has been marketed. The results of future clinical trials may show that our product candidates cause SAEs or undesirable side effects, which could interrupt, delay or halt clinical trials resulting in delay of or failure to obtain marketing approval from the FDA and other comparable foreign regulatory authorities.

If any of our product candidates cause SAEs or undesirable side effects or suffer from quality control issues:

- regulatory authorities may impose a clinical hold or REMS, or comparable foreign regulatory strategies, which could result in substantial delays, significantly increase the cost of development, and/or adversely impact our ability to continue development of the product;
- regulatory authorities may require the addition of statements, specific warnings, or contraindications to the product label, or restrict the product's indication to a smaller potential treatment population;
- we may be required to change the way the product is administered or conduct additional clinical trials;
- we may be required to implement a risk minimization action plan, which could result in substantial cost increases and have a negative impact on our ability to commercialize the product;
- we may be required to limit the participants who can receive the product;
- we may be subject to limitations on how we promote the product;
- we may, voluntarily or involuntarily, initiate field alerts for product recall, which may result in shortages;
- sales of the product may decrease significantly;
- regulatory authorities may require us to take our approved product off the market;

- we may be subject to litigation or product liability claims, and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase commercialization costs and expenses, which in turn could delay or prevent us from generating significant revenues from the sale of our products.

Even if approved, our products will be subject to extensive post-approval regulation.

Once a product is approved, numerous post-approval requirements apply. Among other things, the holder of an approved NDA is subject to periodic and other monitoring and reporting obligations by the FDA, including obligations to monitor and report adverse events and instances of the failure of a product to meet the specifications in the NDA. Application holders must submit new or supplemental applications and obtain the FDA's approval for certain changes to the approved product, product labeling, or manufacturing process. Application holders must also submit advertising and other promotional material to the FDA and report on ongoing clinical trials.

Depending on the circumstances, failure to meet these post-approval requirements can result in criminal prosecution, fines, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of pre-marketing product approvals or refusal to allow us to enter into supply contracts, including government contracts. In addition, even if we comply with the FDA's and others' requirements, new information regarding the safety or effectiveness of a product could lead the FDA to modify or withdraw product approval. Equivalent requirements and penalties are provided in the EU both at EU level and at national level in individual EU Member States.

Even if we obtain regulatory approval to market our product candidates, our product candidates may not be accepted by the market.

Even if the FDA or a comparable foreign regulatory authority approves one or more of our product candidates, physicians and patients may not accept it or use it. Even if physicians and patients would like to use our products, our products may not gain market acceptance among healthcare payors such as managed care formularies, insurance companies or government programs such as Medicare or Medicaid or comparable foreign programs. Acceptance and use of our products will depend upon a number of factors including perceptions by members of the health care community, including physicians, about the safety and effectiveness of our drug or device product; cost-effectiveness of our product relative to competing products; availability of reimbursement for our product from government or other healthcare payors; and effectiveness of marketing and distribution efforts by us and our licensees and distributors, if any.

The degree of market acceptance of any pharmaceutical product that we develop will depend on a number of factors, including:

- cost-effectiveness;
- the safety and effectiveness of our products, including any significant potential side effects as compared to alternative products or treatment methods;
- the timing of market entry as compared to competitive products;
- the rate of adoption of our products by doctors and nurses;
- product labeling or product insert required by the FDA and comparable foreign regulatory authorities for each of our products;
- reimbursement policies of government and third-party payors and the willingness of patients to pay out of pocket in the absence of adequate third-party payor coverage and reimbursement;

- effectiveness of our sales, marketing and distribution capabilities and the effectiveness of such capabilities of our collaborative partners, if any; and

- unfavorable publicity concerning our products or any similar products.

Because we expect sales of our current product candidates, if approved, to generate substantially all of our product revenues for the foreseeable future, the failure of these products to find market acceptance would harm our business and require us to seek additional financing which may not be available.

Risks Related to Our Financial Condition and Capital Requirements; Competition

We will need to raise additional capital which may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates, and additional capital may not be available on favorable terms or at all which may force us to delay, reduce the scope of or eliminate our research and development programs, reduce our commercialization efforts or curtail our operations.

To develop and bring our product candidates to market, we must commit substantial resources to costly and time-consuming research, preclinical studies and clinical trials and marketing activities. Until such time, if ever, as we can generate substantial product revenue, we expect to seek additional funding to meet our operational needs and capital requirements. While we believe that our existing cash and cash equivalents will enable us to fund our operating expenses and capital expenditure requirements into the third quarter of 2025, 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, including if our business or operations change in a manner that consumes available resources more rapidly than we anticipate. Our requirements for additional capital will depend on many factors including:

- changes in direction of our research and development programs;

- the time and expense for preclinical studies and clinical trials for our product candidates;

- the time and costs involved in obtaining regulatory approval for our product candidates;

- the cost increases and other potential impacts of macroeconomic factors, including heightened inflation and rising interest rates, liquidity concerns at and failures of banks and other financial institutions, exchange rate fluctuations, supply chain disruptions and increases in commodity, energy and fuel prices, costs associated with protecting our intellectual property rights;

- successful commercialization of our product candidates;

- competitive and technical advances;

- patent development or regulatory changes;

- development of marketing and sales capabilities;

- payments received under current and future collaboration agreements, if any; and

- market acceptance of our products.

Our ability to continue operations after our current cash resources are exhausted depends on our ability to obtain additional financing or to achieve profitable operations, as to which no assurances can be given. Cash requirements may vary materially from those now planned because of changes in direction of our research and development programs, competitive and technical advances, patent developments, regulatory changes or other developments. If additional sources of financing are not available on favorable terms, or at all, including as a result of actions taken by central banks to counter inflation, volatility in the capital markets, liquidity concerns at and failures of banks and other financial institutions and related market uncertainty, or if we are unsuccessful in entering into partnership agreements for further development of our pipeline, management may need to curtail our development efforts and planned operations to conserve cash.

We expect to finance our operations through a combination of equity offerings, debt financings, government or private party grants, collaborations, strategic alliances and licensing arrangements. We currently have on file with the SEC a shelf registration statement on Form S-3 which allows us to offer and sell our registered common stock, preferred stock, debt securities and or warrants from time to time pursuant to one or more offerings at prices and terms to be determined at the time of sale. On September 6, 2024, we entered into an Equity Distribution Agreement (the "Distribution Agreement") with Oppenheimer & Co. Inc. ("Oppenheimer"), pursuant to which, from time to time, we may offer and sell through Oppenheimer up to $50.0 million of the common stock registered under the shelf registration statement pursuant to one or more "at the market" offerings. From time to time, we have issued and sold shares of common stock pursuant to this agreement and as of December 31, 2024, we have $47 million of common stock remaining available for sale under the Distribution Agreement. Sales of our common stock under the Distribution Agreement with Oppenheimer could be subject to business, economic or competitive uncertainties and contingencies, many of which may be beyond our control, and which could cause actual results from the sale of our common stock to differ materially from expectations. To the extent additional capital is raised through the sale and issuance of shares or other securities convertible into shares, the ownership interest of our stockholders will be diluted. Future issuances of our common stock or other equity securities, or the perception that such sales may occur, could adversely affect the trading price of our common stock and impair our ability to raise capital through future offerings of shares or equity securities. No prediction can be made as to the effect, if any, that future sales of common stock or the availability of common stock for future sales will have on the trading price of our common stock.

We do not currently have any other committed external sources of funds. To the extent we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interest will or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights as a common stockholder. In addition, if we obtain debt financing, a substantial portion of our operating cash flow may be dedicated to the payment of principal and interest on such indebtedness, thus limiting funds available for our business activities. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates, grant licenses on terms that may not be favorable to us or commit to future payment streams.

We face intense competition in the markets targeted by our product candidates. Many of our competitors have substantially greater resources than we do and we expect that all our product candidates under development will face intense competition from existing or future drugs.

We expect that all our product candidates under development, if approved, will face intense competition from existing and future drugs marketed by large companies. These competitors may successfully market products that compete with our products, successfully identify product candidates or develop products earlier than we do, or develop products that are more effective, have fewer side effects or cost less than our products.

Additionally, if a competitor receives FDA approval before we do for a drug that is similar to one of our product candidates, FDA approval for our product candidate may be precluded or delayed due to periods of non-patent exclusivity and/or the listing with the FDA by the competitor of patents covering its newly-approved drug product. Periods of non-patent exclusivity for new versions of existing drugs can extend up to three and one-half years.

In the EU, following grant of a related marketing authorization, innovative medicinal products generally benefit from eight (8) years of data exclusivity and ten years of market exclusivity. Data exclusivity, if granted, prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic application or biosimilar application for eight (8) years from the date of authorization of the innovative product. After this period, an application for marketing authorization for a generic or biosimilar product may be submitted, and the innovator's data may be referenced. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until ten (10) years have elapsed from the initial marketing authorization of the reference product in the EU. The overall ten-year period may occasionally be extended for a further year to a maximum of eleven (11) years if, during the first (8) eight years following authorization of the reference product, 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. There is, however, no guarantee that a product will be considered by the EU's regulatory authorities to be a new chemical/biological entity and therefore products may not qualify for data

exclusivity. In the EU there is also a special regime for biosimilars, or biological medicinal products that are similar to a reference medicinal product but that do not meet the definition of a generic medicinal product. For such products, the results of appropriate preclinical or clinical trials must be provided in support of a related application for marketing authorization. Guidelines from the EMA detail the type and quantity of supplementary data to be provided for different types of biological product.

These competitive factors could require us to conduct substantial new research and development activities to establish new product targets, which would be costly and time consuming. These activities would adversely affect our ability to commercialize products and achieve revenue and profits.

Competition and technological change may make our product candidates and technologies less attractive or obsolete.

We compete with companies that are pursuing other forms of treatment for the same or similar indications we are pursuing including established pharmaceutical and biotechnology companies and that have greater financial and other resources. While we are not currently aware of any other companies that are taking the same therapeutic approach to protein folding disorders to the ones we are pursuing, we are aware of companies developing products for the same target indications. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors.

Other companies may succeed in developing products earlier than us, obtaining FDA or comparable foreign regulatory authority approval for products more rapidly or developing products that are more effective than our product candidates. Research and development by others may render our technology or product candidates obsolete or noncompetitive or result in treatments or cures superior to any therapy we develop. For example, other companies may succeed in developing a technology that addresses protein misfolding and proves to be more effective or is more readily accepted than STARs. We face competition from companies that internally develop competing technology or acquire competing technology from universities and other research institutions. As these companies develop their technologies, they may develop competitive positions that may prevent, make futile or limit our product commercialization efforts which would result in a decrease in the revenue we would be able to derive from the sale of any products.

We may not be able to obtain marketplace acceptance for any of our product candidates as readily as these or other competing treatments. Furthermore, if our competitors' products are approved before ours, it could be more difficult for us to obtain approval from the FDA or comparable foreign regulatory authorities and if they are commercialized before ours they may establish a strong market position before we are able to enter the market. Even if our products are successfully developed and approved for use by all governing regulatory bodies, physicians and patients may not accept our products as a treatment of choice.

The pharmaceutical research industry is diverse, complex and rapidly changing and inherently involves significant and numerous business risks. The effects of competition, intellectual property disputes, market acceptance and FDA and comparable foreign regulatory authority regulations, among other factors described herein, preclude us from forecasting revenues or income with certainty or even confidence.

Our business and operations may be adversely affected by health epidemics or pandemics.

Our business and operations may be adversely affected by pandemics or epidemics, including business interruptions caused by travel restrictions, quarantines, "stay-at-home" and "shelter-in-place" orders, shutdowns requested or mandated by governmental authorities or staffing shortages while employees quarantine as a result of exposure to or transmission of the virus. In addition, health epidemics or pandemics could cause significant disruption in the operations of third-party manufacturers, CROs and other parties upon whom we rely. For example, the COVID-19 pandemic presented a substantial public health and economic challenge around the world and affected employees, patients, communities and business operations as well as the global economy and financial markets.

The COVID-19 pandemic and the resulting post-pandemic environment impacted clinical site activation and patient enrollment. Clinical trial sites experienced limited capacity and staffing shortages in a post-COVID-19 environment, partially due to personnel having been reassigned during the pandemic, resulting in a backlog of patient enrollment and delayed site initiations across the industry. Our inability to successfully recruit and retain patients and principal investigators and site staff in these circumstances could adversely impact our expected future clinical trial operations.

Risks Related to Our Intellectual Property

We rely on a license to use the technology that is material to our business and if the agreements underlying the licenses were to be terminated or if other rights that may be necessary for commercializing our intended products cannot be obtained, it would halt our ability to market our products and technology as well as have an immediate material adverse effect on our business, operating results and financial condition.

We are significantly dependent upon our license with Minoryx Therapeutics S.L. (the "Minoryx License"), as described in the section "Business – Strategic Transactions; Collaboration and Licensing Arrangements – Minoryx Therapeutics, S.L." in our Annual Report. The Minoryx License grants us exclusive, worldwide rights to certain patents and related intellectual property. If we breach the terms of the Minoryx License, for example, by failing to comply with any material terms thereof, Minoryx may have the right to terminate the license. If we were to lose our license under this agreement, we would not be able to market certain of our product candidates and technology which would likely require us to cease our current operations and have an immediate material adverse effect on our business, operating results and financial condition.

Our success depends substantially upon our ability to obtain and maintain intellectual property protection relating to our products and technologies.

We are currently seeking patent protection for numerous compounds and methods of treating diseases. There is no assurance that these patents will be issued and no assurance that, if they do issue, they will prevent other companies from competing with us. Our ability to obtain and enforce patents that may issue from any pending or future patent applications is uncertain and involves complex legal, scientific and factual questions. Thus, we cannot be sure that any patents will issue from any pending or future patent applications owned by or licensed to us. Even if patents do issue, we cannot be sure that the claims of these patents will be held valid or enforceable by a court of law, will provide us with any significant protection against competing products or will afford us a commercial advantage over competitive products. If, at some point in the future, one or more products resulting from our product candidates is approved for sale by the FDA and we do not have adequate intellectual property protection for those products, competitors could duplicate them for approval and sale in the U.S. without repeating the extensive testing required of us to obtain FDA approval.

If we fail to protect our intellectual property rights, our ability to pursue the development of our technologies and products would be negatively affected.

Our success will depend in part on our ability to obtain, maintain and protect intellectual property rights related to our product candidates. If we do not adequately maintain or protect our intellectual property, competitors may be able to use our technologies to produce and market drugs in direct competition with us and erode our competitive advantage. Furthermore, some foreign countries lack rules and methods for defending intellectual property rights and do not protect proprietary rights to the same extent as the U.S. Many companies have had difficulty protecting their proprietary rights in these foreign countries. For example, the legal systems in India, China and certain other developing countries do not favor the enforcement of patents and other intellectual property rights. We may not be able to prevent misappropriation of our proprietary rights and intellectual property rights in these and other countries.

In addition, the patent process is subject to numerous risks and uncertainties and we may not be successful in protecting our products by obtaining and defending patents related to them. These risks and uncertainties include the following: patents that may be issued or licensed may be challenged, invalidated, or circumvented, or otherwise may not provide us any competitive advantage; our competitors, many of which have substantially greater resources than we and many of which have made significant investments in competing technologies, may seek, or may already have obtained, patents that will limit, interfere with, or eliminate our ability to make, use, and sell our potential products either in the U.S. or in international markets; there may be significant pressure on the U.S. government and other international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for treatments that prove successful as a matter of public policy regarding worldwide health concerns; and countries other than the U.S. may have less robust patent laws than those upheld by U.S. courts, allowing foreign competitors the ability to exploit these laws to create, develop, and market competing products using our technologies and patents.

Moreover, any patents issued to us may not provide us with meaningful protection or others may challenge, circumvent or narrow our patents. Third parties may also independently develop products similar to our products, duplicate our unpatented products or design around any patents or proprietary technologies on products we develop. Additionally, extensive time is required for development, testing and regulatory review of a potential product. While extensions of patent terms due to regulatory delays may be available, it is possible that, before any of our product candidates can be commercialized, any related patent, even with an extension, may expire or remain in force for only a short period following commercialization thereby reducing any advantages to us of the patent.

In addition, the U.S. Patent and Trademark Office ("PTO") and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the innovations specifically exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Thus, even if we or our licensors are able to obtain patents, the patents may be substantially narrower than anticipated which could deprive us of rights necessary for the successful commercialization of our product candidates.

Our success depends on our patents and patent applications that may be licensed exclusively to us and other patents and patent applications to which we may obtain assignment or licenses. We may not be aware, however, of all patents, published applications or published literature that may affect our business either by blocking our ability to commercialize our product candidates, by preventing the patentability of our product candidates by us or our licensors or by covering the same or similar technologies. These patents, patent applications and published literature may limit the scope of our future patent claims or adversely affect our ability to market our product candidates. We have not conducted any formal search of patents issued to third parties, and third-party patents containing claims covering our product candidates that predate our patents may exist. Because of the number of patents issued and patent applications filed in our technical areas or fields, our competitors or other third parties may assert that our product candidates are covered by U.S. or foreign patents held by them.

In addition to patents, we rely on a combination of trade secrets, confidentiality, nondisclosure and other contractual provisions and security measures to protect our confidential and proprietary information. These measures may not adequately protect our trade secrets or other proprietary information. If they do not adequately protect our rights, third parties could use our technology and we could lose any competitive advantage we may have. In addition, others may independently develop similar proprietary information or techniques or otherwise gain access to our trade secrets which could impair any competitive advantage we may have.

Patent protection and other intellectual property protection is crucial to the success of our business and prospects and there is a substantial risk that such protections may prove inadequate.

We may be involved in lawsuits to protect or enforce our patents, which could be expensive and time consuming.

The pharmaceutical industry has been characterized by extensive litigation regarding patents and other intellectual property rights and companies have employed intellectual property litigation to gain a competitive advantage. We may become subject to infringement claims or litigation arising out of present and future patents and other proceedings of our competitors. The defense and prosecution of intellectual property suits are costly and time-consuming to pursue, divert the attention of our management and scientific personnel and their outcome is uncertain. Litigation may be necessary to determine the enforceability, scope and validity of the proprietary rights of others. An adverse determination in litigation to which we may become a party could subject us to significant liabilities, require us to obtain licenses from third parties or restrict or prevent us from selling our products in certain markets. Although patent and intellectual property disputes might be settled through licensing or similar arrangements, the costs associated with such arrangements may be substantial and could include paying large, fixed payments and ongoing royalties. Furthermore, the necessary licenses may not be available on satisfactory terms or at all.

Competitors may infringe our patents and we may file infringement claims to counter infringement or unauthorized use. Third parties may assert that our patents are invalid and/or unenforceable in these proceedings. Such litigation can be expensive, particularly for a company of our size, and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is not valid or is unenforceable or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover its technology. An adverse determination of any litigation or defense proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly.

Third parties may also assert that our patents are invalid in patent office administrative proceedings. These proceedings include oppositions in the European Patent Office as well as *inter partes* review and post-grant review proceedings in the PTO. The success rate of these administrative challenges to patent validity in the U.S. is higher than it is for validity challenges in litigation.

Interference or derivation proceedings brought before the PTO may be necessary to determine priority of inventions disclosed in our patents or patent applications. Determining whether a product infringes a patent, as well as priority of inventions and other patent-related disputes, involves complex legal and factual issues and the outcome is often uncertain. During these proceedings, it may be determined that we do not have priority of invention for one or more aspects in our patents or patent applications which could result in the invalidation in part or whole of a patent or could put a patent application at risk of not issuing. Even if successful, an interference or derivation proceeding may result in substantial costs and distraction to our management.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or interference or derivation proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If investors or securities analysts perceive these results to be negative, the price of our common stock could be adversely affected.

Also, a third party may assert that our patents are invalid or unenforceable. There are not currently any unresolved communications, allegations, complaints or threats of litigation that claim our patents are invalid or unenforceable. Any litigation or claims against us, whether or not merited, may result in substantial costs, place a significant strain on our financial resources, divert the attention of management and harm our reputation. An adverse decision in litigation or administrative proceedings could result in inadequate protection for our product candidates and/or reduce the value of any license agreements we have with third parties.

If we infringe the rights of third parties, we could be prevented from selling products or forced to pay damages and defend against litigation.

If our products, methods, processes and other technologies infringe the proprietary rights of other parties, we could incur substantial costs and we may have to obtain licenses, which may not be available on commercially reasonable terms or at all; abandon an infringing product candidate; redesign our products or processes to avoid infringement; stop using the subject matter claimed in the patents held by others; pay damages; and/or defend litigation or administrative proceedings which may be costly whether we win or lose and could result in a substantial diversion of our financial and management resources.

In addition, because patent applications can take many years to issue and publication schedules for pending applications vary by jurisdiction, there may be applications now pending of which we are unaware and may result in issued patents that our future products would infringe. Also, because the claims of published patent applications can change between publication and patent grant, there may be published patent applications that may ultimately issue with claims that we infringe.

We have licensed certain rights, assets and technology related to the Magellan™ platform from Minoryx and we believe that they owned all such rights prior to our license. Although, to our knowledge, no third party has asserted a claim of infringement or other claim against us, others may hold or claim to hold proprietary or other rights that could prevent our Magellan™ platform from being developed or marketed. Any legal action against us claiming damages and seeking to enjoin commercial activities relating to our Magellan™ platform or our processes could subject us to potential liability for damages and require us to obtain a license to continue to manufacture or market any future product candidates based upon the Magellan™ platform. We may not prevail in any such actions and any license required under any of these patents may not be made available on commercially acceptable terms, if at all. In addition, we may not be able to redesign any future product candidates or processes to avoid infringement, if necessary. Accordingly, an adverse determination in a judicial or administrative proceeding or the failure to obtain necessary licenses could prevent us from developing and commercializing our future product candidates which could harm our business, financial condition and operating results.

Risks Related to Third Parties and Collaborators

We currently rely on, and intend to rely on in the future, third parties to conduct, supervise and monitor our clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We currently rely on, and expect to rely on in the future, CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that our clinical trials are conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the CROs will not relieve us of our regulatory responsibilities.

We and our CROs are required to comply with the FDA's Good Clinical Practices ("GCPs") and foreign equivalents 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 clinical trial participants are protected. The FDA and comparable foreign regulatory authorities enforce these GCPs through periodic inspections of study sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving any marketing applications. Upon inspection, the FDA or comparable foreign regulatory authorities may determine that our clinical trials did not comply with applicable GCPs requirements. In addition, our clinical trials will require enrollment and participation of a sufficiently large number of patients to evaluate the effectiveness and safety of our product candidates. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of participants, our clinical trials may be delayed or we may be required to repeat such clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and we are not able to control whether or not they devote sufficient time and resources to our clinical trials. These CROs 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 harm our competitive position.

If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for such product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

We currently rely on and intend to rely in the future on third parties to manufacture the compounds used in our studies, and we intend to rely on them for the manufacture of any approved products for commercial sale. If these third parties do not manufacture our product candidates in sufficient quantities and at an acceptable cost, clinical development and commercialization of our product candidates could be delayed, prevented or impaired.

We have no manufacturing facilities and we intend to rely on third-party contract manufacturing organizations ("CMOs") to manufacture some or all of our product candidates in future clinical trials and our products that reach commercialization. Initiation and completion of our clinical trials and commercialization of our product candidates requires the manufacture of a sufficient supply of our product candidates. If, for any reason, we become unable to rely on these third parties for the manufacture of our product candidates, either for clinical trials or, in the event any of our product candidates are approved, for commercial quantities, then we would need to identify and contract with additional or replacement third-party manufacturers to manufacture compounds for preclinical, clinical and commercial purposes, which we may not be able to do on reasonable terms or at all, or we may be forced to manufacture the materials ourselves, for which we may not have the capabilities or resources. In either scenario, our clinical trials supply could be delayed significantly as we establish alternative supply sources.

We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidates according to any specifications previously submitted to the FDA or another comparable foreign regulatory authority. The delays associated with the verification of a new CMO could negatively affect our ability to develop product candidates or commercialize our products in a timely manner or within budget. Furthermore, a CMO may possess technology related to the manufacture of our product

candidate that such CMO owns independently. This would increase our reliance on such CMO or require us to obtain a license from such CMO in order to have another CMO manufacture our product candidates. In addition, changes in manufacturers often 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. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials.

We believe that there are a variety of manufacturers that we may be able to retain to produce these products. However, we may be in competition with other companies for access to these manufacturers' facilities and may be subject to delays in manufacture if the manufacturers give other clients higher priority than they give to us. If we are unable to secure and maintain third-party manufacturing capacity, the development and sales of our products and our financial performance may be materially affected. In addition, once we retain a manufacturing source, if our manufacturers do not perform in a satisfactory manner, we may not be able to develop or commercialize potential products as planned. Certain specialized manufacturers are expected to provide us with modified and unmodified pharmaceutical compounds, including finished products, for use in our preclinical studies and clinical trials. Some of these materials are available from only one supplier or vendor. Any interruption in or termination of service by such sole source suppliers could result in a delay or interruption in manufacturing until we locate an alternative source of supply. Any delay or interruption in our future supply chain and manufacturing operations (or failure to locate a suitable replacement for such suppliers) as a result of pandemics or epidemics, global geopolitical conflicts or broader global supply chain disruptions, may affect their ability to deliver products to us in a timely manner and, could materially adversely affect our business, prospects, or results of operations. For example, supply chain issues occurred as a result of the COVID-19 pandemic and may continue to occur due to the war between Ukraine and Russia, the conflict between Hamas and Israel and any sanctions resulting therefrom, and global geopolitical tension, including as a result of impacts on energy availability and prices and natural materials availability and prices. We also have a third-party manufacturer in China, which may be impacted by heightened tensions between the United States and China. If we fail to contract for manufacturing on acceptable terms or if third-party manufacturers do not perform as we expect, our development programs could be materially adversely affected. This may result in delays in filing for and receiving FDA or comparable foreign regulatory authority approval for one or more of our products or prevent such approval entirely. Any such delays or failures to obtain regulatory approval could cause our prospects to suffer significantly.

Failure by our third-party manufacturers to comply with the regulatory guidelines set forth by the FDA or comparable foreign regulatory authorities with respect to our product candidates could delay or prevent the completion of clinical trials, the approval of any product candidates or the commercialization of our products.

Third-party manufacturers must be inspected by the FDA and comparable foreign regulatory authorities for cGMP compliance before they can produce commercial products.

Manufacturers are obligated to operate in accordance with requirements mandated by the FDA or comparable foreign regulatory authorities. A failure of any of our third-party manufacturers to establish and follow cGMP requirements and to document their adherence to such practices may lead to significant delays in the availability of material for clinical trials, may delay or prevent filing or approval of marketing applications for our products, and may cause delays or interruptions in the availability of our products for commercial distribution following approval by the FDA or a comparable foreign regulatory authority. This could result in higher costs to us or deprive us of potential product revenues.

Drug manufacturers are subject to ongoing periodic unannounced inspections by the FDA, the Drug Enforcement Administration ("DEA") and corresponding state and foreign regulatory authorities to monitor and ensure strict compliance with cGMP requirements and other requirements under federal drug laws, other government regulations and corresponding foreign laws, regulations and standards. If we or our third-party manufacturers fail to comply with applicable regulations, sanctions could be imposed on us, including fines, injunctions, civil penalties, failure by the government or competent regulatory authorities to grant marketing approval of drugs, delays, suspension, variation or withdrawal of approvals, seizures or recalls of product, shutdown of the manufacturer, invalidation of drug lots or processes, operating restrictions, product recalls and criminal prosecutions.

Corporate and academic collaborators may take actions to delay, prevent, or undermine the success of our products.

Our operating and financial strategy for the development, clinical testing, manufacture, and commercialization of product candidates is heavily dependent on our entering into collaborations with corporations, academic institutions, licensors, licensees, and other parties and we may not be successful in establishing such collaborations. Some of our existing collaborations are, and future collaborations may be, terminable at the sole discretion of the collaborator. Replacement collaborators might not be available on attractive terms, or at all. The activities of any collaborator will not be within our control and may not be within our power to influence. Any collaborators may not perform their obligations to our satisfaction, or at all, we may not derive any revenue or profits from such collaborations, and any collaborators may ultimately compete with us. If any collaboration is not pursued, we may require substantially greater capital to undertake development and marketing of our proposed products and may not be able to develop and market such products effectively, if at all. In addition, a lack of development and marketing collaborations may lead to significant delays in introducing proposed products into certain markets and/or reduced sales of proposed products in such markets.

Changes in U.S. and international trade policies, particularly with respect to China, may adversely impact our business and operating results.

One of our primary manufacturers and suppliers, WuXi AppTec ("WuXi"), is located in China and the subject of increased U.S. government scrutiny. Trade tensions and conflicts between the United States and China have been escalating in recent years and, as such, we are exposed to the possibility of product and material supply disruption and increased costs and expenses in the event of changes to the laws, rules, regulations and policies of the governments of the United States or China, or due to geopolitical unrest and unstable economic conditions.

The U.S. government has recently made statements and taken certain actions that may lead to potential changes to U.S. and international trade policies, including imposing several rounds of tariffs and export control restrictions affecting certain products manufactured in China. Increased focus on relations with China has included U.S. legislative proposals, such as the proposed BIOSECURE Act, which has been passed by the U.S. House of Representatives and is pending before the U.S. Senate. If enacted, the BIOSECURE Act would, among other things, prohibit U.S. federal agencies from entering into or renewing any contract with any entity that uses biotechnology equipment or services produced or provided by a "biotechnology company of concern" to perform that contract with the government. Although the proposed BIOSECURE Act has not been enacted and thus is subject to change through the legislative process, a version of the BIOSECURE Act passed by the U.S. House of Representatives defines a "biotechnology company of concern" to include WuXi. If adopted, the BIOSECURE Act could cause us to seek to exit some or all of our arrangements with WuXi (or any other China-based service provider determined to be "biotechnology companies of concern") and accelerate the transition of these services to alternative companies or continue to engage redundant suppliers for the U.S. market. Additionally, the legislation could adversely impact WuXi's operations or financial position which, in turn, could impact its ability to perform under our agreements with it. Our reliance on Chinese-based contract research organizations, such as WuXi, may also cause us to face additional risks due to geopolitical tensions between the U.S. and China and related legal and regulatory restrictions and requirements, including measures directly affecting WuXi.

Any unfavorable government policies on international trade, such as export controls, capital controls or tariffs, may increase the cost of manufacturing our product candidates and platform materials, affect the demand for our drug products (if and once approved), the competitive position of our product candidates, and import or export of raw materials and finished product candidate used in our and our collaborators' preclinical studies and clinical trials, particularly with respect to any product candidates and materials that we import from China, including pursuant to our arrangements with WuXi. If any new tariffs, export controls, legislation and/or regulations are implemented, or if existing trade agreements are renegotiated or, in particular, if either the U.S. or Chinese government takes retaliatory trade actions due to the recent trade tension, such changes could have an adverse effect on our business, financial condition and results of operations. Any negative impact of the ability of our third party collaborators to deliver the materials we require to conduct our clinical operations due to political actions, supply chain disruptions or otherwise, may have a material adverse impact on our results of operations or financial condition.

Data provided by collaborators and others upon which we rely that has not been independently verified could turn out to be false, misleading, or incomplete.

We rely on third-party vendors, scientists and collaborators to provide us with significant data and other information related to our projects, clinical trials and our business. If such third parties provide inaccurate, misleading or incomplete data, our business, prospects and results of operations could be materially adversely affected.

If we fail to establish marketing, sales and distribution capabilities, or fail to enter into arrangements with third parties, we will not be able to create a market for our product candidates.

General Risk Factors

As a public company, we are obligated to develop and maintain proper and effective controls over financial reporting. If we fail to maintain proper and effective system of internal controls over financial reporting in the future, 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 securities.

Our management is responsible for establishing and maintaining an adequate system of internal control over financial reporting, as defined in Rule 13a-15(f) under the Exchange Act. In addition, Section 404 of the Sarbanes-Oxley Act of 2002 ("Section 404") and related SEC rules require management to furnish a report on the effectiveness of our internal control over financial reporting. Effective internal controls are necessary for us to provide reliable financial reports and help us to prevent fraud. The process of implementing our internal controls and complying with Section 404 is expensive and time consuming and requires significant continuous attention of management. We cannot be certain that these measures will ensure that we maintain adequate controls over our financial processes and reporting in the future.

If we fail to maintain the adequacy of our internal controls, including any failure to implement new or improved controls or if we experience difficulties in their implementation, our business and financial results could be harmed and we would be required to disclose material weaknesses in future filings with the SEC, which could adversely affect our business, investor confidence in our company and the market price of our common stock and could subject us to litigation or regulatory enforcement actions. As a result, shareholders could lose confidence in our financial and other public reporting, which would harm our business and the market value of our common stock.

Global and macroeconomic conditions, including economic, political and social instability, could adversely affect our financial condition or results of operations.

The global credit and financial markets have recently experienced extreme volatility and disruptions including severely diminished liquidity and credit availability, disruptions in access to bank deposits and lending commitments due to bank failures, declines in economic growth, increases in unemployment rates, supply chain disruptions, heightened interest rates and inflation, stock volatility and uncertainty about economic stability. Such conditions may continue or worsen in the future. The financial markets and the global economy may also be adversely affected by the current or anticipated impact of military conflict including Russia's invasion of Ukraine and the conflict between Hamas and Israel, terrorism or other geopolitical events. Sanctions imposed by the U.S. and other countries in response to such conflicts, including sanctions imposed in connection with the war in Ukraine and the conflict between Hamas and Israel, the effect of tariffs and/or any resulting trade wars, increasing interest rates, or other factors may also adversely impact the financial markets and the global economy and any economic countermeasures by affected countries and others could exacerbate market and economic instability. For example, in late 2024 and early 2025, the United States, Canada, China, and the European Union each announced either new tariffs, non-tariff barriers, or export controls. Any of these risks, ensuing retaliation, or the further deterioration of trade relations between countries could have an adverse impact on our financial condition and results of operations. Additional tariffs or further retaliatory trade measures taken by China or other countries in response could affect the demand for any of our products, impact the competitive position of our products, prevent us from being able to sell products in certain countries or otherwise adversely impact our results of operations. Growing tensions, protectionist trade policies, and tariffs may also lead to a fragmentation of the global economy, a general reduction of international trade in goods and services, and a reduction in the integration of financial markets, any of which could materially and adversely affect our financial condition, or prospects. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur.

Our general business strategy as well as our suppliers' ability to provide us with raw materials and components, may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions, which could directly affect our ability to attain our operating goals on schedule and on budget, including requiring us to delay or abandon certain development plans and could have a material adverse effect on our growth strategy, financial performance and stock price. In addition, there is a risk that one or more of our current suppliers may not survive an economic downturn, which could directly affect our ability to attain our operating goals on schedule and within budget.

Changes in trade policies, including the imposition of tariffs or other trade restrictions, could materially impact our ability to obtain the raw materials, active pharmaceutical ingredients, and other components necessary for the manufacturing of our product candidates used in our clinical development activities. Some of these materials may be sourced from foreign suppliers, and any increase in tariffs or duties on imported goods could significantly raise the cost of doing business. Additionally, retaliatory tariffs, trade disputes, trade wars, or changes in international trade agreements may lead to supply chain disruptions, including delays in obtaining critical components or the need to seek alternative suppliers. If we are unable to mitigate the impact of increased costs or supply chain disruptions, our financial condition, and ability to develop our product candidates in a timely manner, could be adversely affected.

We will need to expand our operations and increase the size of our company, and we may experience difficulties in managing growth.

As we advance our product candidates through preclinical studies and clinical trials and develop new product candidates, we will need to increase our product development as well as our scientific, regulatory and compliance and administrative headcount to manage these programs. In addition, to continue to meet our obligations as a public company, and particularly when we no longer qualify as an emerging growth company, we will need to increase our general and administrative capabilities. Our management, personnel and systems currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and various projects requires that we:

- successfully attract and recruit new employees with the expertise and experience we will require;
- manage our clinical programs effectively, which we anticipate being conducted at numerous clinical sites;
- develop a marketing, distribution and sales infrastructure in addition to a post-marketing surveillance program if we seek to market our products directly; and
- continue to improve our operational, manufacturing, quality assurance, financial and management controls, reporting systems and procedures.

If we are unable to successfully manage this growth and increased complexity of operations, our business may be adversely affected.

We depend upon our key personnel and our ability to attract and retain qualified employees.

Our future growth and success will depend in large part on our continued ability to attract, retain, manage and motivate our employees. The loss of the services of a significant portion of our workforce or any member of our senior management or the inability to hire or retain qualified personnel could adversely affect our ability to execute our business plan and harm our operating results.

Because of the specialized nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. In particular, the loss of one or more of our senior executive officers could be detrimental to us if we do not have an adequate succession plan or if we cannot recruit suitable replacements in a timely manner. While our senior executive officers are parties to employment agreements with us, these agreements do not guarantee that they will remain employed with us in the future. In addition, our arrangements with our senior executive officers include only limited, if any, restrictions on our senior executive officers' ability to compete with us after their employment is terminated.

The competition for qualified personnel in the pharmaceutical field is intense and there is a limited pool of qualified potential employees. Due to the intense competition for talent, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. We may also face increased costs in attracting and retaining personnel as a result of heightened global inflation.

To incentivize valuable employees to join and remain at our company, in addition to salary and other employee benefits, we have provided stock option and restricted stock unit awards that vest over time and in some instances, subject to the achievement of performance milestones. The value to employees of such awards may be significantly affected by movements in our stock price and current market conditions and extreme stock price volatility may diminish our ability to incentivize employees through the use of such awards.

If we are unsuccessful in our recruitment and retention efforts, our business may be adversely affected.

Under applicable employment laws, we may not be able to enforce covenants not to compete and therefore may be unable to prevent our competitors from benefiting from the expertise of some of our former employees.

Our employment arrangements generally include covenants not to compete. These agreements prohibit our employees, if they cease working for us, from competing directly with us or working for our competitors for a limited period. We may be unable to enforce these agreements under the laws of the jurisdictions in which our employees work at all or for a sufficient duration of time to prevent members of our management team from competing with us. If we are unable to enforce these covenants not to compete, we may be unable to prevent our competitors from benefiting from the expertise of our former employees or consultants and our competitiveness may be diminished.

Our relationships with customers, physicians, and third-party payors will be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws and other healthcare laws and regulations including comparable foreign laws and regulations. If we are unable to comply or have not fully complied with such laws, we could face substantial penalties.

Healthcare providers and third-party payors in the U.S. and elsewhere will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors may subject us to various federal and state fraud and abuse laws and other healthcare laws including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims laws and the law commonly referred to as the Physician Payments Sunshine Act and regulations, and foreign equivalent laws and regulations. These laws will impact, among other things, our proposed clinical research, sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct or may conduct our business as well as foreign data privacy and security laws and regulations. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;

- federal civil and criminal false claims laws, including, without limitation, the False Claims Act and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the Health Insurance Portability and Accountability Act ("HIPAA"), which created new federal criminal statutes that prohibit a person from, among other things, knowingly and willfully executing a scheme or making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

- HIPAA, as amended by HITECH and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach

Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and certain health care providers, and their respective business associates and covered subcontractors;

- federal transparency laws, including the federal Physician Payments Sunshine Act, which is part of the Patient Protection and Affordable Care Act ("ACA"), that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services ("CMS"), information related to: (i) payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals; and (ii) ownership and investment interests held by physicians and their immediate family members;

- state and foreign law equivalents of each of the above federal laws, state laws and foreign law equivalents that require manufacturers to report information related to payments and other transfers of value to physicians and other healthcare professionals or marketing expenditures, state laws and foreign law equivalents that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or competent regulatory authority or to adopt compliance programs as prescribed by applicable laws and regulations, or that otherwise restrict payments that may be made to healthcare professionals; and

- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws.

It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid or comparable foreign programs, 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 curtailment or restructuring of our operations.

The risk of us being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts and their provisions are open to a variety of interpretations. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

Coverage and adequate reimbursement may not be available for our current or any future product candidates, which could make it difficult for us to sell profitably, if approved.

Market acceptance and sales of any product candidates that we commercialize, if approved, will depend in part on the extent to which reimbursement for these drugs and related treatments will be available from third-party payors including government health administration authorities, managed care organizations and other private health insurers. Third-party payors decide which therapies they will pay for and establish reimbursement levels. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and

reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any product candidates that we develop will be made on a payor-by-payor basis. One payor's determination to provide coverage for a drug does not determine whether another payor will also provide coverage and adequate reimbursement for the drug. Additionally, a third-party payor's decision to provide coverage for a therapy does not imply that an adequate reimbursement rate will be approved. Each payor determines whether it will provide coverage for a therapy, what amount it will pay the manufacturer for the therapy and on what tier of its formulary it will be placed. The position on a payor's list of covered drugs, or formulary, generally determines the co-payment that a patient will need to make to obtain the therapy and can strongly influence the adoption of such therapy by patients and physicians. Even if favorable coverage and reimbursement status is attained for any product candidate for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our drugs unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of our drugs.

Outside the U.S., reimbursement and healthcare payment systems vary significantly by country and many countries have instituted price ceilings on specific products and therapies. For example, the EU provides options for EU Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. An EU Member State may approve a specific price for the medicinal product, refuse to reimburse a product at the price set by the manufacturer or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Many EU Member States also periodically review their reimbursement procedures for medicinal products which could have an adverse impact on reimbursement status. We expect that legislators, policymakers and healthcare insurance funds in the EU Member States will continue to propose and implement cost-containing measures such as lower maximum prices, lower or lack of reimbursement coverage and incentives to use cheaper, usually generic, products as an alternative to branded products and/or branded products available through parallel import to keep healthcare costs down.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Coverage and reimbursement may not be available for any drug that we commercialize and, if reimbursement is available, it is uncertain what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for or the price of any drug for which we obtain marketing approval. If coverage and adequate reimbursement are not available or are available only at limited levels, we may not be able to successfully commercialize our current and any future product candidates that we develop.

Healthcare legislative reform measures may have a negative impact on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval.

Among policy makers and payors in the U.S. and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the U.S., the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, the Affordable Care Act ("ACA") of 2010 substantially changed the way healthcare is financed by both the government and private insurers and significantly impacts the U.S. pharmaceutical industry. The ACA has been subject to judicial and Congressional challenges but remains in place for all intents and purposes. The Inflation Reduction Act of 2022 ("IRA"), which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program, it also allows the U.S. government to negotiate Medicare Part B and Part D pricing for certain high-cost drugs and biologics without generic or biosimilar competition, require companies to pay rebates to Medicare for drug prices that increase faster than inflation, and delay the rebate rule that would require pass through of pharmacy benefit manager rebates to beneficiaries. The implementation of the IRA is currently subject to ongoing litigation challenging the constitutionality of the IRA's Medicare drug price negotiation program. The effect of IRA on our business and the healthcare industry

in general is not yet known. With the recent change in administration, the future of the IRA and its effects remain uncertain.

Other legislative changes have been proposed and adopted since the ACA was enacted including aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2032 unless additional Congressional action is taken.

Moreover, changes to the political landscape in the United States may impact the market sentiment surrounding the pharmaceutical industry. Since retaking office, President Trump has signed several Executive Orders that may impact the health and pharmaceutical industry. On January 20, 2025, President Trump began the action of withdrawing the United States from the World Health Organization. This order also rescinded a prior executive order signed by formed President Biden that coordinated the federal government's COVID-19 response efforts and implemented processes to respond to emerging pandemics. In addition, President Trump has proposed reductions in federal research spending that may impact organizations such as the National Institutes of Health, the National Science Foundation and the Centers for Disease Control and Prevention.

Funding from government agencies and reimbursement programs such as the NIH, Medicare and Medicaid, including the overall availability and reimbursement rates under these programs, often fluctuates and is subject to the political process, which is often unpredictable. For example, on February 7, 2025, the NIH issued Notice Number NOT-OD-25-068, a guidance document pronouncing that reimbursement for certain indirect costs would be capped at 15% for existing and future grant recipients, a rate that is lower than the in-place rate for many existing grant recipients. Certain of our third party collaborators may depend on NIH grants and reimbursements to partially fund research. Any reduction in the availability or rate of funding or reimbursement, or delays surrounding the approval of such funding or reimbursement, may adversely impact our ability to develop our product candidates. The outcome of these orders is, and will likely remain, uncertain for the foreseeable future.

In addition, to obtain reimbursement for our products in some European countries including some EU Member States, we may be required to compile additional data comparing the cost-effectiveness of our products to other available therapies. The Health Technology Assessment, or HTA, of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States including those representing the larger markets. The HTA process is the procedure to assess therapeutic, economic and societal impact of a given medicinal product in the national healthcare systems of the individual country. The outcome of an HTA will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States. In December 2021, Regulation No 2021/2282 on HTA, amending Directive 2011/24/EU, was adopted in the EU. This Regulation, which entered into force in January 2022 will apply as of January 2025. It is intended to boost cooperation among EU Member States in assessing health technologies, including new medicinal products, and providing the basis for cooperation at EU level for joint clinical assessments in these areas. The Regulation will permit EU Member States to use common HTA tools, methodologies, and procedures across the EU to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU Member States will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technologies, and making decisions on pricing and reimbursement. If we are unable to maintain favorable pricing and reimbursement status in EU Member States for product candidates that we may successfully develop and for which we may obtain regulatory approval, any anticipated revenue from and growth prospects for those products in the EU could be negatively affected.

We expect that these and other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and additional downward pressure on the price that we receive for any approved drug. Any reduction in reimbursement from Medicare or other comparable foreign programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs.

If we obtain approval to commercialize any approved products outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If any of our product candidates are approved for commercialization outside of the United States, we intend to enter into agreements with third parties to market them on a worldwide basis or in more limited geographical

regions. We expect that we will be subject to additional risks related to entering into international business relationships, including:

- different regulatory requirements for drug approvals;
- reduced protection for intellectual property rights including trade secret and patent rights;
- unexpected changes in tariffs, export controls, sanctions, trade barriers and regulatory requirements;
- economic weakness including inflation or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes including withholding of taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- potential noncompliance with the U.S. Foreign Corrupt Practices Act, the U.K. Bribery Act 2010 or similar anti-bribery and anticorruption laws in other jurisdictions;
- business interruptions resulting from geopolitical actions including war (such as Russia's invasion of Ukraine and the conflict between Hamas and Israel) and terrorism, natural disasters such as earthquakes, hurricanes, floods and fires, economic or political instability, sanctions, or public health emergencies, and related shelter-in-place orders, travel, social distancing and quarantine policies, boycotts, curtailment of trade and other business restrictions; and
- difficulty in importing and exporting clinical trial materials and study samples.

We are subject to U.S. and certain foreign anti-corruption, anti-money laundering, export and import controls, and sanctions laws and regulations. Non-compliance with such laws can subject us to criminal and/or civil liability and harm our business.

We are subject to the U.S. Foreign Corrupt Practices Act of 1977, as amended, or FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act and anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents and contractors from authorizing, promising, offering or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. We may have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. In addition, we may engage third-party intermediaries to promote our clinical research activities and/or to obtain necessary permits, licenses and other regulatory approvals. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors or other partners even if we do not explicitly authorize or have actual knowledge of such activities.

We are also subject to export control and import laws and regulations including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls. Export controls and trade sanctions laws and regulations may restrict or prohibit altogether the provision, sale, or supply of our product candidates to certain

governments, persons, entities, countries and territories including those that are the target of comprehensive sanctions or an embargo.

We cannot ensure that all our employees, agents, contractors or those of our affiliates will comply with all applicable laws and regulations. Violations of anti-corruption, anti-money laundering, import and export control or sanctions laws and regulations could result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, breach of contract and fraud litigation, reputational harm and other consequences.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.

We will face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and will face an even greater risk if we commercialize any of our product candidates. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- injury to our reputation and significant negative media attention;
- initiation of investigations by regulators;
- withdrawal of clinical trial participants;
- significant time and expenses to defend the related litigation;
- diversion of management and scientific resources from our business operations;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any product candidates that we may develop.

We currently hold limited product liability insurance coverage. We will need to purchase additional product liability insurance coverage as we expand our clinical trials, and if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. If we are unable to obtain insurance at an acceptable cost or otherwise protect against potential product liability claims, we will be exposed to significant liabilities which may materially and adversely affect our business and financial position. If we are sued for any injury allegedly caused by our company's or our collaborators' products, our liability could exceed our total assets and our ability to pay the liability. A product liability claim or series of claims brought against us would decrease our cash and could cause our stock price to fall.

We are subject to stringent and evolving U.S. and foreign laws, regulations, rules, contractual obligations, policies, contractual and other obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation (including class actions) and mass arbitration demands; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences.

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal information and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, and sensitive third-party data. Our data processing activities may subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contracts, and other obligations that govern the processing of personal information by us and on our behalf.

In the U.S., federal, state, and local governments have enacted numerous data privacy and security laws including data breach notification laws, personal information privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act) and other similar laws (e.g., wiretapping laws). For example, HIPAA, as amended by HITECH, imposes specific requirements relating to the privacy, security and transmission of protected

health information. Several states have also enacted comprehensive data privacy laws, which either became effective in 2023 or will become effective within the next couple of years. These state comprehensive data privacy laws provide individuals with certain rights concerning their personal information including the right to access, correct or delete certain personal information and opt-out of certain data processing activities such as targeted advertising, profiling, and automated decision-making. One example of these comprehensive state data privacy laws is the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 ("CPRA") (collectively, "CCPA") which applies to the personal information of consumers, business representatives and employees who are California residents. It requires businesses to provide specific disclosures in privacy notices and honor requests of such California residents to exercise certain rights related to their personal information such as those noted above. The CCPA provides for administrative fines for noncompliance (up to $7,500 per violation) and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA exempts some data processed in the context of clinical trials, the CCPA increases compliance costs and potential liability with respect to other personal information we maintain about California residents. In addition, the CPRA expanded the CCPA's requirements, including by adding a new right for individuals to correct their personal information and establishing a new regulatory agency, the California Privacy Protection Agency, to implement and enforce the law. These new comprehensive data privacy laws (including the CCPA) and individuals' exercise of their rights under these laws may impact our business and ability to provide our products and services. In addition, other data privacy and security laws have been proposed and others have been passed at the federal, state and local levels in recent years. While some of these laws exempt data processed in the context of clinical trials, these developments may nonetheless further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties upon whom we rely.

Outside the U.S., an increasing number of laws, regulations and industry standards apply to data privacy and security. For example, the European Union's General Data Protection Regulation (the "EU GDPR") and the United Kingdom GDPR (the "UK GDPR") (collectively, the "GDPR") impose strict requirements for processing personal information and violators of these laws face significant penalties. For example, under the GDPR, government regulators may impose temporary or definitive bans on data processing as well as fines of up to 20 million euros under the EU GDPR (17.5 million British Pounds under the UK GDPR) or 4% of annual global revenue, in either case, whichever is greater, or we may be subject to private litigation related to processing of personal information brought by classes of data subjects or consumer protection organizations authorized at law to represent. In addition, the Swiss Federal Act on Data Protection (the "FADP") also applies to the collection and processing of personal information including health-related information, by companies located in Switzerland, or in certain circumstances, by companies located outside of Switzerland. The FADP has been revised and adopted by the Swiss Parliament. Companies must comply with the revised version of the FADP and its revised ordinances which may result in an increase of costs of compliance, risks of noncompliance and penalties for noncompliance.

In the ordinary course of business, we may transfer personal information from Europe and other jurisdictions to the U.S. or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal information to other countries. In particular, the European Economic Area (the "EEA"), the UK and Switzerland have significantly restricted the transfer of personal information to the U.S. and other countries whose privacy laws they generally believe are inadequate. Other jurisdictions may adopt similarly stringent interpretations of their data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal information from the EEA and UK to the U.S. in compliance with law such as the EEA and UK's standard contractual clauses, the UK's International Data Transfer Agreement / Addendum and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers for relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal information to the U.S. If there is no lawful manner for us to transfer personal information from the EEA, the UK or other jurisdictions to the U.S., or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties and injunctions against our processing or transferring of personal information necessary to operate our business. Some European regulators have prevented companies from transferring personal information out of Europe for allegedly violating the EU GDPR's cross-border data transfer limitations. For example, in May 2023, the Irish Data Protection Commission determined that a major social media company's use of the standard contractual clauses to transfer personal data from Europe to the U.S. was insufficient and levied a 1.2 billion Euro fine against the company and prohibited the company from transferring personal data to the U.S.

Our employees and personnel may use generative artificial intelligence ("AI") technologies to perform their work and the disclosure and use of personal information in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions and consumer lawsuits. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages.

In addition to data privacy and security laws, we are contractually subject to industry standards adopted by industry groups and may become subject to such obligations in the future. We are also bound by other contractual obligations related to data privacy and security and our efforts to comply with such obligations may not be successful.

Furthermore, we publish privacy policies, marketing materials and other statements such as compliance with certain certifications or self-regulatory principles, regarding data privacy and security. If these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences.

Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing in an increasingly stringent fashion creating some uncertainty as to the effective future legal framework. Additionally, these obligations may be subject to differing applications and interpretations which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires significant resources and may necessitate changes to our information technologies, systems and practices and to those of any third parties that process personal information on our behalf. Although we endeavor to comply with all applicable data privacy and security obligations, we may at times fail, or be perceived to have failed, to do so. Moreover, despite our efforts, our personnel or third parties upon whom we rely, may fail to comply with such obligations which could negatively impact our business operations and compliance posture. For example, any failure by a third-party processor to comply with applicable law, regulations or contractual obligations could result in adverse effects and proceedings against us by governmental entities or others.

If we or the third parties upon which we rely fail, or are perceived to have failed, to address or comply with data privacy and security obligations, we could face significant consequences. These consequences may include, but are not limited to, government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class actions) and mass arbitration demands; additional reporting requirements and/or oversight; bans on processing personal information; orders to destroy or not use personal information; and imprisonment of company officials. Plaintiffs have become increasingly more active in bringing privacy-related claims against companies including class actions and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis and, if viable, carry the potential for monumental statutory damages depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business or financial condition including but not limited to the loss of customers; interruptions or stoppages in our business operations (including, as relevant, clinical trials); inability to process personal information or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or revision or restructuring of our operations.

Issues relating to the use of AI and machine learning could adversely affect our business and operating results.

Magellan™ is our platform technology that leverages AI-supported structural biology, proprietary algorithms and physics-based models powered by the cutting-edge CSCS Swiss National Supercomputing Centre to explore novel allosteric binding pockets on disease-implicating proteins. Issues relating to the use of new and evolving technologies such as AI and machine learning may cause us to experience brand or reputational harm, competitive harm, legal liability and new or enhanced governmental or regulatory scrutiny. We may incur additional costs to resolve such issues. As with many innovations, AI presents risks and challenges that could undermine or slow its adoption and therefore harm our business. For example, perceived or actual technical, legal, compliance, privacy, security, ethical or other issues relating to the use of AI may cause public confidence in AI to be undermined, which could harm our business reputation. In addition, litigation or government regulation related to the use of AI may also adversely impact our and others' abilities to develop and offer products that use AI, as well as increase the cost and complexity of doing so. Developing, testing and deploying AI systems may also increase the cost profile of our product offerings due to the nature of the computing costs involved in such systems, which could impact our project margin and adversely affect our business and operating results. Further, market demand and acceptance of AI technologies are uncertain and we may be unsuccessful in our product development efforts.

If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse consequences.

In the ordinary course of our business, we or the third parties upon which we rely process proprietary, confidential, and sensitive data, including personal information (such as health-related data), business plans, financial information, intellectual property, and trade secrets (collectively, sensitive information), and, as a result, we and the third parties upon which we rely face a variety of evolving threats.

Cyberattacks, malicious internet-based activity, online and offline fraud, and other similar activities are prevalent and continue to increase. These threats are becoming increasingly difficult to detect. These threats come from a variety of sources, including traditional computer "hackers," threat actors, "hacktivists," organized crime threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyber-attacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, including the war in Ukraine and the conflict between Hamas and Israel, we and the third parties upon which we rely may be vulnerable to a heightened risk of these attacks, including cyber-attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services.

We and the third parties upon which we rely are subject to a variety of evolving threats, including but not limited to social-engineering attacks (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks, credential stuffing, credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, attacks enhanced or facilitated by AI, and other similar threats. In particular, ransomware attacks, including by organized criminal threat actors, nation-states, and nation-state-supported actors, are becoming increasingly prevalent and severe and can lead to significant interruptions in our operations, ability to provide our products or services, loss of data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

Remote work has become more common and has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers, and devices outside our premises or network, including working from home, while in transit and in public locations. Future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

In addition, our reliance on third-party service providers could introduce new cybersecurity risks and vulnerabilities, including supply-chain attacks, and other threats to our business operations. We rely upon third-party service providers and technologies to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, third-party providers of cloud-based infrastructure, encryption and authentication technology, employee email, content delivery to customers, and other functions. We also rely on third-party service providers to provide other products, services, or otherwise to operate our business. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. Additionally, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties and infrastructure in our supply chain or our third-party partners' supply chains have not been compromised or that they do not contain exploitable defects or bugs that could result in a breach of or disruption to our information technology systems or the third-party information technology systems that support us and our services.

Any of the previously identified or similar threats could cause a security incident or other interruption. A security incident or other interruption could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information. A security incident or other interruption could disrupt our ability (and that of third parties upon whom we rely) to operate our business.

We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations may require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information. While we and our third-party service providers have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps to detect and remediate vulnerabilities in our information technology systems, but we may not be able to detect and remediate all vulnerabilities because the threats and techniques used to exploit the vulnerability change frequently and are often sophisticated in nature. Therefore, such vulnerabilities could be exploited but may not be detected until after a security incident has occurred. Un-remediated high risk or critical vulnerabilities pose material risks to our business. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities.

Applicable data privacy and security obligations may require us to notify relevant stakeholders of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences. If we (or a third party upon whom we rely) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences. These consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal information); litigation (including class actions); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); financial loss; and other similar harms.

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 data privacy and security obligations. In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, our sensitive Company information could be leaked, disclosed, or revealed as a result of or in connection with our employees', personnels', or vendors' use of generative AI technologies.

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.

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. For example, on March 10, 2023, Silicon Valley Bank ("SVB") was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation ("FDIC") as receiver. Similarly, on March 12, 2023, Signature Bank and Silvergate Capital Corp. were each swept into receivership. Future adverse developments with respect to specific financial institutions or the broader financial services industry may lead to market-wide liquidity shortages, impair our ability to access near-term working capital needs, and create additional market and economic uncertainty. There can be no assurance that future credit and financial market instability and a deterioration in confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, liquidity shortages, volatile business environment or continued unpredictable and unstable market conditions. If the equity and credit markets deteriorate, or if adverse developments are experienced by financial institutions, it may cause short-term liquidity risk and make any necessary debt or equity financing more difficult, more costly, more onerous with respect to financial and operating covenants and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, financial institutions, manufacturers, and

other partners may be adversely affected by the foregoing risks, which could directly affect our ability to attain our operating goals on schedule and on budget.

In addition, any further deterioration in the macroeconomic 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 projected business operations and results of operations and financial condition.

Risks Related to Ownership of Our Common Stock

The market price for our common stock has been and likely will continue to be volatile, and your investment in our securities could decline in value.

Our stock price has been highly volatile and is likely to continue to be so. The stock market in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of the issuer. The market price for our common stock may be influenced by many factors, including:

- results from, and any delays in our preclinical studies and any other clinical development programs, including any delays related to the health epidemics or pandemics or other factors outside of our control;
- actual or anticipated changes in estimates as to financial results, development timelines and other company milestones or recommendations by securities analysts;
- announcements of changes to our operational focus, including changes to the programs we are actively developing;
- announcements by our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- announcements of technological innovations or new products by us or our competitors;
- announcement of FDA or comparable foreign regulatory authority approval or disapproval of our product candidates or other product-related actions;
- developments involving our discovery efforts and clinical trials;
- developments or disputes concerning patents or proprietary rights, including announcements of infringement, interference or other litigation against us or our potential licensees;
- developments involving our efforts to commercialize our products, including developments impacting the timing of commercialization;
- announcements concerning our competitors, or the biotechnology, pharmaceutical or drug delivery industry in general;
- public concerns as to the safety or efficacy of our product candidates or our competitors' products;
- changes in government regulation of the pharmaceutical or medical industry;
- changes in the reimbursement policies of third-party insurance companies or government agencies;
- actual or anticipated fluctuations in our operating results;
- changes in financial estimates or recommendations by securities analysts;
- developments involving corporate collaborators, if any;

- changes in accounting principles;
- general economic, industry and market conditions, exchange rate fluctuations, supply chain disruptions and fluctuating commodity, energy and fuel prices;
- the impact of political instability, natural disasters, events of terrorism and/or war, such as the war in Ukraine and the conflict between Hamas and Israel, and the corresponding tensions created from such conflict between Russia, the United States and countries in Europe as well as other countries such as China; and
- the loss of any of our key scientific or management personnel.

In the past, securities class action litigation has often been brought against companies that experience volatility in the market price of their securities and in particular, biotechnology and pharmaceutical companies. Whether meritorious, litigation brought against us could result in substantial costs and a diversion of management's attention and resources which could adversely affect our business, operating results and financial condition.

Stock market volatility and declines in the price of our common stock also increase the likelihood that we may fail to meet the minimum bid price requirement of $1.00 for continued listing on the Nasdaq Global Market. If the Nasdaq Global Market delists our securities from trading on its exchange for failure to meet the listing standards, we and our stockholders could face significant negative consequences, including:

- limited availability of market quotations for our securities;
- a determination that the common stock is a "penny stock" which will require brokers trading in the common stock to adhere to more stringent rules, possibly resulting in a reduced level of trading activity in the secondary trading market for shares of common stock;
- a limited amount of analyst coverage; and
- a decreased ability to issue additional securities or obtain additional financing in the future.

We incur and will continue to incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we will no longer qualify as an emerging growth company, we incur and will continue to incur significant legal, accounting and other expenses. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq, and other applicable securities rules and regulations impose various requirements on U.S. reporting public companies, including the establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. These rules and regulations are often subject to varying interpretations and as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

While we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, pursuant to Section 404 of the Sarbanes-Oxley Act, we are required to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. The process to document and evaluate our internal control over financial reporting is both costly and challenging. In this regard, we need to continue to dedicate internal resources, validate through testing that controls are functioning as designed and maintain a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could

result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We are an "emerging growth company," and the reduced reporting requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We qualify as an "emerging growth company," as defined in the JOBS Act. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to public companies that are not emerging growth companies. These provisions include, but are not limited to, being permitted to report only two years of audited financial statements and only two years of related selected financial data and management's discussion and analysis of financial condition and results of operations disclosure; an exemption from compliance with the auditor attestation requirement in the assessment of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act; reduced disclosure obligations regarding executive compensation arrangements in our periodic reports, registration statements and proxy statements; and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, the JOBS Act permits emerging growth companies to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. As a result, the information we provide might differ from the information that is available for other public companies. We cannot predict whether investors will find our common stock less attractive if we 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 the market price of our common stock may be more volatile.

We will remain an emerging growth company until the earliest of (i) December 31, 2026, (ii) the first fiscal year after our annual gross revenue exceeds $1.235 billion, (iii) the date on which we have, during the immediately preceding three-year period, issued more than $1.00 billion in non-convertible debt securities, or (iv) the end of any fiscal year in which the market value of our common stock held by non-affiliates exceeds $700 million as of the end of the second quarter of that fiscal year.

We are currently listed on the Nasdaq Global Market. If we are unable to maintain listing of our securities on the Nasdaq Global Market or any stock exchange, our stock price could be adversely affected and the liquidity of our stock and our ability to obtain financing could be impaired and it may be more difficult for our stockholders to sell their securities.

Although our common stock is currently listed on the Nasdaq Global Market, we may not be able to continue to meet the exchange's minimum listing requirements or those of any other national exchange. If we are unable to maintain listing on the Nasdaq or if a liquid market for our common stock does not develop or is sustained, our common stock may remain thinly traded.

The Listing Rules of the Nasdaq require listing issuers to comply with certain standards in order to remain listed on its exchange. If, for any reason, we should fail to maintain compliance with these listing standards and the Nasdaq should delist our securities from trading on its exchange and we are unable to obtain listing on another national securities exchange, a reduction in some or all of the following may occur, each of which could have a material adverse effect on our stockholders:

- the liquidity of our common stock;
- the market price of our common stock;
- our ability to obtain financing for the continuation of our operations;
- the number of institutional and general investors that will consider investing in our common stock;
- the number of investors in general that will consider investing in our common stock;
- the number of market makers in our common stock;
- the availability of information concerning the trading prices and volume of our common stock; and

- the number of broker-dealers willing to execute trades in shares of our common stock.

In the past, we have received notices from the Nasdaq's Listing Qualifications Department indicating that we had not complied with certain of the Nasdaq Global Market's continued listing standards. While we have regained compliance for each instance, there can be no assurance that we will continue to maintain compliance with the Nasdaq listing requirements. A delisting could substantially decrease trading in our common stock, adversely affect the market liquidity of our common stock as a result of the loss of market efficiencies associated with the Nasdaq and the loss of federal preemption of state securities laws, adversely affect our ability to obtain financing on acceptable terms, if at all, and may result in the potential loss of confidence by investors, suppliers, and employees and lead to fewer business development opportunities. Additionally, the market price of our common stock may decline further, and stockholders may lose some or all of their investment.

In the event of a delisting, we anticipate that we would take actions to restore our compliance with the Nasdaq Global Market or another national exchange's listing requirements, but we can provide no assurance that any such action taken by us would allow our common stock to remain listed on the Nasdaq Global Market, stabilize our market price, improve the liquidity of our common stock, prevent our common stock from dropping below the Nasdaq Global Market's minimum bid price requirement, or prevent future non-compliance with the Nasdaq Global Market or another national exchange's listing requirements.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

As of December 31, 2024, we had net operating loss, or NOL, carryforwards of approximately $47 million. We have incurred substantial losses during our history, do not expect to become profitable in the foreseeable future and may never achieve profitability. Net operating losses of our Swiss subsidiary can be carried forward for up to seven years and will begin to expire commencing from 2025 for the NOLs generated in 2018 under applicable Swiss tax law. Under applicable U.S. federal income tax law, our federal NOL carryforwards generated in tax years beginning on or before December 31, 2017, are only permitted to be carried forward for 20 years. Our federal NOL carryforwards generated in tax years beginning after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOL carryforwards may be limited. Similar provisions of state tax law may also apply to limit the use of any state NOLs. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited.

We have experienced ownership changes in the past. Utilization of the net operating loss carryforwards may be subject to an annual limitation due to ownership changes that may have occurred previously. Such annual limitation could result in the expiration of net operating losses and credits before their utilization. We have not yet completed a Section 382 analysis, and therefore, there can be no assurances over any previous ownership changes and their impacts on our ability to utilize loss carryforwards. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which are outside of our control. As a result, if we earn net taxable income, our ability to use our pre-change NOL carryforwards to offset taxable income may be limited, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOL carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed by us.

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flow, financial condition, or results of operations.

New tax laws, statutes, rules, regulations, or ordinances could be enacted at any time. For instance, the IRA imposes, among other rules, a 15% minimum tax on the book income of certain large corporations and a 1% excise tax on certain corporate stock repurchases. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted differently, changed, repealed, or modified at any time. Any such enactment, interpretation, change, repeal, or modification could adversely affect us, possibly with retroactive effect. In particular, changes in corporate tax rates, the realization of our net deferred tax assets, the taxation of foreign earnings, and the deductibility of expenses under the Tax Cuts and Jobs Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act or any

future tax reform legislation, could have a material impact on the value of our deferred tax assets, result in significant one-time charges, and increase our future tax expenses.

We do not anticipate paying dividends on our common stock and, accordingly, stockholders must rely on stock appreciation for any return on their investment.

We have never declared or paid cash dividends on our common stock and do not expect to do so in the foreseeable future. The declaration of dividends is subject to the discretion of our board of directors and limitations under applicable law and will depend on various factors including our operating results, financial condition, future prospects and any other factors deemed relevant by our board of directors. You should not rely on an investment in our company if you require dividend income. The success of your investment will likely depend entirely upon any future appreciation of the market price of our common stock which is uncertain and unpredictable. There is no guarantee that our common stock will appreciate in value.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly. We cannot predict what effect, if any, sales of our shares in the public market or the availability of shares for sale will have on the market price of our common stock. However, future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options, or the perception that such sales may occur, could adversely affect the market price of our common stock.

We also expect that significant additional capital may be needed in the future to continue our research and development activities and costs associated with operating as 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. 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.

The rights of the holders of our securities may be impaired by the potential issuance of preferred stock.

Our amended and restated certificate of incorporation (the "Amended Charter") gives our board of directors the ability to designate and issue preferred stock in one or more series. As a result, the board of directors may, without stockholder approval, issue preferred stock with voting, dividend, conversion, liquidation or other rights which could adversely affect the relative voting power and equity interest of the holders of common stock. Preferred stock, which could be issued with the right to more than one vote per share, could have the effect of discouraging, delaying or preventing a change of control of us. The possible impact on takeover attempts could adversely affect the price of our securities. Although we have no present intention to designate any series, or issue any shares, of preferred stock, other than pursuant to the IPO, we may do so in the future.

If securities or industry analysts do not publish research or reports about our business, or if they change their recommendations regarding our stock adversely, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. Our research coverage by industry and financial analysts is currently limited. Even if our analyst coverage increases, if one or more of the analysts who cover us downgrade our stock, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail 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.

Anti-takeover provisions in our organizational documents and Delaware law might discourage or delay attempts to acquire us that you might consider favorable.

Our Amended Charter and amended and restated bylaws (the "Amended Bylaws") contain provisions that may make the merger or acquisition of us more difficult without the approval of our board of directors. Among other things, these provisions:

- allow us to authorize the issuance of undesignated preferred stock in connection with a stockholder rights plan or otherwise, the terms of which may be established and the shares of which may be issued without stockholder approval, and which may include super voting, special approval, dividend, or other rights or preferences superior to the rights of the holders of common stock;

- provide that our bylaws may be amended or repealed only by a majority vote of our board of directors or by the affirmative vote of the holders of at least 66 2/3% of the votes which all our stockholders would be entitled to cast in any annual election of directors; and

- establish advance notice requirements for nominations for elections to our board of directors or for proposing matters that can be acted upon by stockholders at stockholder meetings.

Further, as a Delaware corporation, we are also subject to provisions of Delaware law which may impair a takeover attempt that our stockholders may find beneficial. These anti-takeover provisions and other provisions under Delaware law could discourage, delay or prevent a transaction involving a change in control, including actions that our stockholders may deem advantageous or could negatively affect the market price of our common stock. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing and to cause us to take other corporate actions our stockholders desire.

Our Amended Charter provides that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for substantially all disputes between us and our stockholders and federal district courts will be the sole and exclusive forum for Securities Act claims, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our Amended Charter provides that, unless we consent to the selection of an alternative forum, the Court of Chancery of the State of Delaware is the sole and exclusive forum for: (i) any derivative action or proceeding brought on our behalf; (ii) any action asserting a claim of breach of fiduciary duty owed by any of our directors, officers, or other employees to us or to our stockholders; (iii) any action asserting a claim arising pursuant to the Delaware General Corporation Law (the "DGCL"), the Amended Charter or the Amended Bylaws or as to which the DGCL confers exclusive jurisdiction on the Court of Chancery of the State of Delaware; or (iv) any action asserting a claim governed by the internal affairs doctrine, provided that the exclusive forum provisions will not apply to suits brought to enforce any liability or duty created by the Securities Exchange Act of 1934, as amended, or the Exchange Act or to any claim for which the federal courts have exclusive jurisdiction. Our Amended Charter further provides that, unless we consent in writing to the selection of an alternative forum, the federal district courts are the sole and exclusive forum for the resolution of any complaint asserting a right under the Securities Act, subject to a final adjudication in the State of Delaware of the enforceability of such exclusive forum provision. We note that investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder. The choice of 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 such lawsuits against us and our directors, officers, and other employees. Alternatively, if a court were to find the choice of forum provisions contained in our Amended Charter to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business, results of operations, and financial condition.

Provisions in our organizational documents regarding exculpation and indemnification of our directors and officers may result in substantial expenditures by us and may discourage lawsuits against our directors and officers.

Our Amended Charter and Amended Bylaws provide for the elimination, to the maximum extent permissible under Delaware law, of the personal liability of our directors and officers to us and our stockholders for damages for breach of fiduciary duty. These provisions may discourage us, or our stockholders through derivative litigation, from bringing a lawsuit against any of our current or former directors or officers for any breaches of their fiduciary duties even if such legal actions, if successful, might benefit us or our stockholders. In addition, our Amended Charter and

Amended Bylaws provide that we will, to the fullest extent permitted by Delaware law, indemnify our directors and officers for costs or damages incurred by them in connection with any threatened, pending or completed action, suit or proceeding brought against them by reason of their positions as directors and officers. We also intend to enter into indemnification agreements with each of our directors and executive officers. These indemnification obligations could result in us incurring substantial expenditures to cover the cost of settlement or damage awards against our directors or officers.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 1C. CYBERSECURITY

Risk Management and Strategy

We do no believe that there are currently any known risks from cybersecurity threats that are reasonably likely to materially affect us or our business strategy, results of operations or financial condition. During the reporting period, we have not experienced any material cybersecurity incidents nor any series of immaterial cybersecurity incidents that would require to be disclosed in this year-end report

Management of cybersecurity risks is a component of our overall risk management strategy. We rely on information technology, communication networks, enterprise applications, accounting and financial platforms, and related systems in the operation of its business. Our operations also rely on the secure collection, storage, transmission and processing of proprietary, confidential and sensitive data. Our cybersecurity risk management strategy is designed to support us in identifying, protecting, detecting, responding to, and recovering from cybersecurity threats and incidents with the intention of protecting the confidentiality, integrity, and availability of such systems and data.

We have implemented several processes with the assistance of third parties that we engage to help us manage our overall information technology function. These include certain processes for assessing, identifying and managing cybersecurity risks and are designed to help protect our information assets and operations from internal and external cyber threats, and to protect employee, collaborator and patient information from unauthorized access or attack, as well as to secure our networks and systems. Such processes include physical, procedural and technical safeguards, response plans, regular tests on our systems, and periodic review of our policies and procedures to identify risks and refine our practices. We engage certain external parties, including consultants, computer security firms and governance experts, to enhance our cybersecurity oversight and to gain valuable insights into the ever-evolving cybersecurity landscape.

Our use of third parties to conduct our business is significant. We use suppliers, CROs, CDMOs, and other service providers. A cybersecurity incident at third party could materially adversely impact us. In order to mitigate this risk, we assess third party cybersecurity controls prior to engaging third parties and include security and privacy addendums to our contracts where applicable. We also require that third party service providers or partners report cybersecurity incidents to us so that we can assess the impact of the incident on us.

In an effort to further deter and detect cyber threats, we provide all employees, including part-time and temporary employees, with data protection, cybersecurity and incident response and prevention materials, which educates employees on the importance of reporting all incidents immediately. We also use technology-based tools that are designed to mitigate cybersecurity risks.

Lastly, we have established a Cybersecurity Incident Response Policy ("CIRP"), which details the steps to be followed to properly respond to, contain, and remediate a cybersecurity incident. The CIRP provides a process for escalating certain cybersecurity incidents to the Board and members of management to facilitate management-level consideration as to whether a cybersecurity incident may be material to the Company and whether public disclosure of the incident is required.

Governance

Our third-party service providers report to our Principal Financial Officer who is responsible for the management of the Cybersecurity program. The Principal Financial Officer, on a regular basis, reports to the Audit Committee.

The Audit Committee of our Board of Directors provides direct oversight over cybersecurity risk and provides updates to the Board of Directors regarding such oversight. The Audit Committee receives periodic updates from management regarding cybersecurity matters and is notified between such updates regarding significant new cybersecurity threats or incidents.

ITEM 2. PROPERTIES

Our principal executive office is located in Bethesda, Maryland, where we lease in a multi-tenant building 1,568 square feet of office space that we use for our general management, investor relations, business developments and other activities. This lease expires in September 2025.

We lease 2,992 square feet of office space in a multi-tenant building in Via Soave n.6, Lugano Switzerland that we use for our research and development team and finance and administrative activities. This lease expires in May 2026.

We lease 1,402 square feet that we use for our biology laboratory and 245 square feet that we use for a warehouse space in a multi-tenant building in Cluster II Building in the Parc Cientific de Barcelona, Spain. This lease expires in December 2025.

We lease 1,417 square feet of office space in a multi-tenant building in Torre D Building in the Parc Cientific de Barcelona, Spain that we use for our drug discovery and research activities. This lease expires in November 2026.

We believe our current facilities, including the terms and conditions of the relevant lease agreements, are adequate to operate our businesses as currently conducted. However, as we continue to expand our operations, we may need to lease additional or alternative facilities.

ITEM 3. LEGAL PROCEEDINGS

On September 18, 2024, Matthias Alder ("Mr. Alder") filed suit against us in the Circuit Court of Maryland for Montgomery County (the "Litigation"). On October 10, 2024, Mr. Alder amended the complaint in the Litigation to add Jeffrey Riley, a member of our board of directors, and Khalid Islam, Executive Chairman of the board of directors, as defendants. Mr. Alder served as our Chief Operating Officer and subsequently as Chief Executive Officer during his approximately two and a half year tenure with us beginning in October 2021. Mr. Alder's employment with us was terminated on June 25, 2024. In connection with Mr. Alder's departure, we entered into a separation and general release agreement with Mr. Alder on June 27, 2024 (the "Separation Agreement"). In his suit, Mr. Alder alleges, among other things, that we breached the Separation Agreement and employment agreement with Mr. Alder by failing to pay certain severance amounts as well as violated non-disparagement obligations to Mr. Alder. We are vigorously defending ourselves in the matter. If the lawsuit is not amicably resolved at a mediation session currently scheduled for March 31, 2025, we will consider filing counterclaims against Mr. Alder.

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 and Holders

Our common stock trades on the Nasdaq Global Market (the "Nasdaq") under the trading symbol "GANX." As of February 28, 2025, there were approximately 73 holders of record of our common stock. This number does not reflect the beneficial holders of our common stock for whom shares are held in "nominee" or "street" name through brokerage accounts or other nominees.

Dividends

We have never declared or paid cash dividends on our share capital, and we do not currently intend to pay any cash dividends on our share capital in the foreseeable future. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business. Any future determination related to dividend policy will be made at the discretion of our board of directors, subject to applicable laws, and will depend upon, among other factors, our results of operations, financial condition, contractual restrictions, and capital requirements. In addition, our ability to pay cash dividends on our share capital in the future may be limited by the terms of any future debt or preferred securities we issue or any credit facilities we enter into.

ITEM 6. SELECTED FINANCIAL DATA

Reserved.

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

Overview

We are a biotechnology company developing novel small molecule therapeutics to treat diseases across several therapeutic areas, including, central nervous system ("CNS") disorders, lysosomal storage disorders ("LSDs") and metabolic disorders through molecular chaperoning to stabilize misfolded proteins and increase their activity, as well as other diseases that can be targeted through protein inactivation or modulation, such as oncology. We use our computational target and drug discovery platform, Magellan™, to discover novel allosteric binding sites on proteins implicated in a disease and to identify proprietary small molecules that bind these sites to modulate protein function and treat the underlying cause of the disease. We believe that Magellan™ is uniquely suited to identify allosteric binding sites on the protein surface, which are different from the active (or orthosteric) binding site where the natural ligand of the protein binds. Targeting an allosteric binding site instead of the active binding site of a protein provides numerous advantages, including: the ability to regulate proteins implicated in disease through several different mechanisms of action covering both functional and conformational effects, including stabilization, destabilization, targeted degradation, allosteric inhibition, and allosteric activation of the targeted protein; improved specificity of small molecules because binding to an allosteric binding site is non-competitive with the natural substrate that binds to the active binding site; and the ability to identify small molecules with more favorable drug-like properties. We have used our drug discovery platform to identify novel allosteric sites and small molecules for all of our pipeline programs. We plan to continue to advance our existing research programs and initiate additional programs targeting allosteric binding sites identified with the Magellan™ platform in various therapeutic areas through academic partnerships, co-development and licensing arrangements.

Our clinical stage product candidate, GT-02287, is being developed for the treatment of Parkinson's disease with and without GBA1 mutations. We have generated an extensive preclinical data package providing evidence of the mechanism of action, in vivo pharmacology, and safety of GT-02287. In preclinical models of GBA1 Parkinson's disease, GT-02287 has been shown to restore glucocerebrosidase, or GCase, function in the lysosome, reduce toxic lipid substrates and toxic forms of alpha-synuclein, reduce endoplasmic reticulum stress, improve mitochondrial health and overall survival of dopaminergic neurons, increasing dopamine levels, restoring locomotor and cognitive function, and reducing plasma-based neurodegeneration maker, neurofilament light chain (NfL), back to the level of control animals. In a Phase 1 first-in-human study (n = 72), GT-02287 was safe and generally well tolerated up to and including

the highest planned dose level, enabling further development in GBA1 Parkinson's patients. Additionally, administration of GT-02287 was associated with a mean increase in GCase activity of 53% among healthy volunteers at doses that were predicted to be in the therapeutic range based on preclinical models and will be carried forward in later stage trials of GT-02287. The good safety and tolerability profile and the observed range of plasma exposure levels achieved after oral administration further bolster GT-02287's best in-class potential.

We continue to monitor the impacts on our operations and access to financing, global and worsening macroeconomic conditions, such as the war in Ukraine, the Hamas-Israel conflict, global geopolitical tension, exchange rate fluctuations, supply chain disruptions, liquidity concerns and increases in commodity, energy and fuel prices.

Financial Condition

Since our inception in 2017, we have devoted substantially all of our resources to identify and develop next-generation brain-penetrant allosteric small molecules for the treatment of devastating diseases with high-unmet medical needs using our Magellan™ platform. Our operations have consisted primarily of organizing and staffing the Company, expanding its operations, securing financing, performing research, conducting preclinical studies and acquiring, developing and securing our in-licensed technology. To date, we do not have any product candidates approved for sale and have not generated any revenue from product sales, and as a result, we face risks associated with early-stage biotechnology companies whose product candidates are in development. We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our product candidates. We expect our research and development expenses to remain significant, and to increase to support progress in our research and development activities. In addition, if we obtain regulatory approval for our product candidates and do not enter into a third-party commercialization partnership, we expect to incur significant expenses related to developing our commercialization capability to support product sales, marketing, manufacturing and distribution activities. These efforts require significant amounts of additional capital for us to complete our research and development, achieve our research and development objectives, defend our intellectual property rights, and recruit and retain skilled personnel, and key members of management. Even if our product development efforts are successful, it is uncertain when, if ever, we will realize significant revenue from product sales.

In May 2022, we filed a shelf registration statement on Form S-3, which covers the offering, issuance and sale of up to a maximum aggregate offering price of $100.0 million of any combination of our common stock, preferred stock, debt securities and/or warrants from time to time in one or more offerings.

In the second quarter of fiscal year 2022, we entered into a Controlled Equity Offering Sales Agreement (the "Cantor Sales Agreement"), with Cantor Fitzgerald & Co. ("Cantor"), pursuant to which we were able to offer and sell shares of our common stock having an aggregate offering price of up to $16.0 million from time to time through or to Cantor, acting as our agent or principal, in a series of one or more at-the-market equity offerings. During the year ended December 31, 2023, we sold an aggregate of 862,535 shares of common stock at an average price of $4.60 per share, raising gross proceeds of $3.9 million, which included $0.4 million in sales and commissions and other offering expenses. The Cantor Sales Agreement was terminated in conjunction with the public offering and concurrent private placement of shares of our common stock in November 2023, as described below.

In November 2023, we completed the public offering of 2.5 million shares of our common stock and warrants to purchase 1.3 million shares of our common stock. The warrants have an exercise price of $2.75 per share and were sold at the rate of one warrant for every two shares of common stock purchased in the public offering. The public offering price for each set of two shares of common stock and accompanying warrant to purchase one share of common stock was $4.01 (with an effective price of $2.00 per share and $0.01 per warrant). In a private placement that was completed concurrently with the public offering we also issued to accredited investors 2.5 million of shares of our common stock (or pre-funded warrants in lieu thereof) and private warrants to purchase 2.5 million shares of our common stock with an exercise price of $2.75 per share. The private offering price per share and accompanying warrant in the private placement was $2.00 per set of securities sold privately. The public offering and the concurrent private placement resulted in combined gross proceeds of $10.1 million, which included $1.2 million of underwriting commissions, placement agent's fees and other expenses connected with the financing round. Additionally, a total of 353,156 warrants to purchase an equal amount of our common stock at an exercise price of $2.75 per share were granted to the underwriter and the placement agent associated with the offerings as consideration for the services provided, which provide for cash-less exercise.

In June 2024, we completed the public offering of 7.1 million shares of our common stock and 1.0 million pre-funded warrants (the "Pre-Funded Warrants") to purchase an equal amount of our common stock at the nominal exercise price of $0.0001. The public offering price is $1.35 per share while the purchase price of each pre-funded warrant was equal to the public offering price at which a share of common stock was sold to the public in this offering, minus $0.0001. The public offering resulted in gross proceeds of $11.0 million, which included $1.2 million of underwriting commissions and other expenses connected with the financing round.

As part of the public offering in June 2024, we granted the underwriter an over-allotment option to purchase up to an additional 1,222,222 shares of our common stock, at the public offering price of $1.35, less underwriting discounts and commissions. In July 2024, the underwriter partially exercised the over-allotment option and purchased an additional 337,076 shares of our common stock at the offering price mentioned above. The exercise of the over-allotment option resulted in gross proceeds of $0.46 million, which included $42 thousand of underwriting commissions and other expenses connected with the exercise of the option. We also issued 593,965 warrants to purchase an equal amount of our common stock at an exercise price of $1.6875 per share to the underwriter as consideration for the services provided, which provide for cash-less exercise.

In the third quarter of 2024, we entered into an Equity Distribution Agreement (the "Distribution Agreement") with Oppenheimer & Co. Inc., serving as agent ("Oppenheimer") with respect to an at-the-market ("ATM") offering program (the "2024 ATM Program"). Under the 2024 ATM Program we may offer and sell, from time to time at our sole discretion, shares of our common stock having an aggregate offering price of up to $50.0 million. We will pay Oppenheimer a commission equal to 3.0% of the gross sales proceeds of any shares sold through Oppenheimer under the Distribution Agreement. During the year ended December 31, 2024, we sold an aggregate of 1,597,128 shares of our common stock at a weighted average price of $1.97 per share under the 2024 ATM Program, raising gross proceeds of $3.1 million, which included $0.2 million in sales commissions and other offering expenses.

From inception through December 31, 2024, we have raised an aggregate of $89 million of gross proceeds through equity financing, including the issuance of convertible preferred stock, our initial public offering, secondary offerings and previous sales under our ATM programs.

As of December 31, 2024, we had cash and cash equivalents of $10.4 million. We have incurred recurring losses and negative cash flows from operations since inception and as of December 31, 2024 and December 31, 2023, had an accumulated deficit of $81.2 million and $60.8 million, respectively. We anticipate incurring additional losses until such time, if ever, that we can generate sales of our product candidates currently in development. We have not generated any product revenues and have not achieved profitable operations. There is no assurance that profitable operations will ever be achieved, and, if achieved, could be sustained on a continuing basis. In addition, we will need significant additional financing to fund our operations and to develop our product candidates. Our ability to continue operations after our current cash resources are exhausted depends on our ability to obtain additional financing or to achieve profitable operations, as to which no assurances can be given. Cash requirements may vary materially from those now planned because of changes in direction of our research and development programs, competitive and technical advances, patent developments, regulatory changes or other developments. If adequate additional funds are not available when required, or if we are unsuccessful in entering into partnership agreements for further development of our pipeline, management may need to curtail our development efforts and planned operations to conserve cash.

Going Concern

As of December 31, 2024 and December 31, 2023, we had an accumulated deficit of $81.2 million and $60.8 million, respectively, and as of December 31, 2024, we had cash and cash equivalents of $10.4 million. During the year ended December 31, 2024, we incurred net losses of $20.4 million and negative cash flows from operations of $18.9 million. Our current operating plan indicates that we will continue to incur losses from operations and generate negative cash flows from operating activities. Our projected cash outflows for the upcoming periods raise substantial doubt about our ability to continue as a going concern for at least 12 months from the issuance of the financial statements included elsewhere in this Annual Report. We will need to raise additional capital to fund continued operations beyond the third quarter of 2025. We plan to address our liquidity needs by taking steps to improve our operations and cash position, including identifying access to future capital and potential cost-reduction measures.

Financing Requirements; Current Financing Environment

Until such time, if ever, as we can generate substantial product revenues to support our business and corporate strategy, we expect to finance our cash needs through a combination of public and private equity offerings, including an at-the-market offering, debt financings, government or private party grants, collaborations, strategic alliances and licensing arrangements. We may not be able to obtain financing on acceptable terms, or at all, and we may not be able to enter into strategic alliances or other arrangements on favorable terms, or at all. The terms of any financing may adversely affect our holdings or the rights of our stockholders. If we are unable to obtain funding, we could be required to delay, limit, reduce or eliminate research and development programs, product portfolio expansion or future commercialization efforts, or grant rights to develop, sell and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves, which could adversely affect our business prospects.

The war in Ukraine, the conflict between Hamas and Israel, global geopolitical tensions, and the post COVID-19 environment continue to have unpredictable impacts on global societies, economies, financial markets, and business practices. Recently worsening global macroeconomic conditions, liquidity concerns at and failures of banks and other financial institutions, volatility in the capital markets, and related market uncertainty may impact our ability to obtain additional financing when needed on favorable terms or at all.

Strategic Transactions; Collaboration and Licensing Arrangement

In connection with our business development activities, we are continually looking to enter into collaboration and licensing arrangements with third parties to use our licensed Magellan™ computational platform technology to discover novel allosteric sites on proteins and identify proprietary small molecules that bind these sites and may be developed into pharmaceutical products. We expect to continue to identify and evaluate collaboration, co-development and licensing opportunities that may be similar to or different from the collaboration and licenses arrangements that we have entered into.

Components of Our Consolidated Results of Operations

Revenue

We have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the foreseeable future, if at all. If we fail to complete the development of our product candidates in a timely manner or fail to obtain their regulatory approval and successfully commercialize them, we will not generate revenues in the future. Historically, we have received limited collaboration revenue pursuant to a collaboration agreement with Zentalis Pharmaceuticals which concluded in the year ended December 31, 2023.

Operating Expenses

Our operating expenses since inception have consisted solely of research and development and general and administrative costs.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and the development of our product candidates, which include:

- expenses incurred under collaborations with third parties, including contract research organizations ("CROs") and universities, that conduct research, preclinical and clinical studies, such as in-vitro and in-vivo absorption, distribution, metabolism and excretion ("ADME"), cell model studies, in-vivo pharmacology and pharmacokinetic studies, toxicology studies and chemical synthesis, stability studies, manufacturing and control materials, process characterization, scale-up and transfer, clinical trial expenses, on our behalf;

- employee salaries, benefits and other related costs, including share-based compensation expenses, for employees engaged in research and development functions and overhead allocations consisting of

various support and facilities-related expenses, which include rent, utilities and maintenance of our facilities, depreciation, travel and conference expenses;

- fees paid to consultants who assist with research and development activities and related travel expenses; and
- the cost of sponsored research, which includes laboratory materials and supplies, manufacturing scale-up expenses and the cost of acquiring and manufacturing preclinical studies.

The following table provides a breakout of our research and development expenses by major category:

	Year Ended December 31,		
	2024	2023	Change
Pre-clinical activities, clinical activities and outside services	$ 8,395,066	$ 7,535,538	$ 859,528
Personnel expenses	4,174,178	3,956,187	217,991
Other	369,693	628,958	(259,265)
Research grants	(2,147,879)	(600,070)	(1,547,809)
Total research and development expenses	$ 10,791,058	$ 11,520,613	$ (729,555)

We recognize research and development costs as incurred. We recognize external development costs based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our financial statements as prepaid or accrued research and development expenses. We anticipate that our research and development expenses will increase substantially in future periods to support progress in our research and development activities, including the progressing of the clinical trials for product candidates we are developing. These increases will likely also result from expanded infrastructure and increased insurance costs. Such expenses are offset by contributions from research grants, which are recorded as a reduction to research and development expenses based on our best estimate of the periods in which the related expenditures are incurred and activities performed.

Our primary research and development focus since inception has been the application of our Magellan™ platform to various indications and targets, and more recently the development of our clinical stage lead product candidate GT-02287 for the treatment of Parkinson's disease and other neurodegenerative diseases.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally incur higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect that our research and development expenses will continue to increase in the foreseeable future as we (i) increase personnel costs, including stock-based compensation, (ii) continue preclinical development of our lead compounds, (iii) progress our clinical trials for certain product candidates, (iv) continue to discover and develop additional product candidates, and (v) pursue later stages of clinical development of product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, bonus and other related costs, including share-based compensation, for personnel in our executive, finance, corporate and business development and administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters, professional fees for accounting, auditing, tax and consulting services, insurance costs, travel expenses, and facility-related expenses, and other operating costs.

We will continue to focus on preserving our liquidity resources while we seek to maximize shareholders' value.

Other Financial Income (Expense)

Other financial income (expense) consists of interest income, interest expense and foreign exchange gain or loss, net.

Consolidated Results of Operations

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

	Year Ended December 31,		
	2024	2023	Increase (Decrease)
Revenues:			
Collaboration revenues	$ —	$ 55,180	(55,180)
Other income	—	—	—
Total revenues	—	55,180	(55,180)
Operating expenses:			
Research and development	(10,791,058)	(11,520,613)	(729,555)
General and administrative	(9,559,534)	(10,787,700)	(1,228,166)
Total operating expenses	(20,350,592)	(22,308,313)	(1,957,721)
Loss from operations	(20,350,592)	(22,253,133)	(1,902,541)
Other income (expense):			
Interest income, net	357,096	494,234	(137,138)
Foreign exchange gain (loss), net	119,120	(429,346)	548,466
Loss before income tax	(19,874,376)	(22,188,245)	(2,313,869)
Income tax	(536,815)	(79,275)	457,540
Net loss	$ (20,411,191)	$ (22,267,520)	$ (1,856,329)
Net loss per shares:			
Net loss per share attributable to common stockholders - basic and diluted	$ (0.89)	$ (1.71)	(0.82)
Weighted average common stock - basic and diluted	22,881,415	13,011,361	

Comparison of the Years Ended December 31, 2024 and 2023

Revenues

For the years ended December 31, 2024 and 2023, total revenues were nil and $55 thousand, respectively, and consisted mainly of income from a collaboration agreement with Zentalis Pharmaceuticals that ended as of December 31, 2023.

Research and development expenses

Research and development expenses decreased by $0.7 million to $10.8 million for the year ended December 31, 2024, as compared to $11.5 million for the year ended December 31, 2023. The decrease in research and development expenses was primarily related to higher recognition of research grant income principally comprised of a tax credit for eligible research and development expenses in Australia. These increases were partially offset by higher costs associated with the Clinical Phase 1 trial of our lead program compound GT-02287 for the treatment of Parkinson's disease.

General and administrative expenses

General and administrative expenses decreased by $1.2 million to $9.6 million for the year ended December 31, 2024 from $10.8 million for the year ended December 31, 2023. The decrease in general and administrative expenses was primarily attributable to lower personnel and stock-based compensation costs.

Foreign exchange gain (loss), net

Foreign exchange loss, net decreased by $0.5 million to a gain of $0.1 million for the year ended December 31, 2024 from a loss of $0.4 million for the year ended December 31, 2023. The decrease was mainly attributable to favorable foreign exchange currency translation as the Swiss franc weakened against the U.S. dollar.

Interest income, net

Interest income, net decreased by $0.1 million to $0.4 million for the year ended December 31, 2024 from $0.5 million for the year ended December 31, 2023. The decrease was mainly attributable to lower interest income from treasury securities that reached maturity in April 2024.

Income taxes

Income taxes are $537 thousand and $79 thousand for the years ended December 31, 2024 and 2023, respectively. The increase was mainly attributable to higher income taxes payable in Australia.

Liquidity and Capital Resources

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from our operations. We have not yet received approval for or commercialized any products or technologies, and we do not expect to generate revenue from sales of any products in the near term, if at all. As described in additional detail under "Financial Condition" above, we have funded our operations to date primarily through a combination of sales of our securities and research grants.

As of December 31, 2024 and December 31, 2023, we had $10.4 million in cash and cash equivalents and $16.8 million in cash, cash equivalents and marketable securities, respectively, and an accumulated deficit of $81.2 million and $60.8 million, respectively. We had indebtedness of $0.4 million and $0.6 million as of December 31, 2024 and December 31, 2023, respectively. Our cash and cash equivalents available at December 31, 2024 are expected to be sufficient to fund our anticipated operating and capital requirements into the third quarter of 2025 and will not be sufficient to finance our operations for one year from the issuance of the financial statements included in this Annual Report. Therefore, we have reported that there is substantial doubt about our ability to continue as a going concern. Please refer to the discussion above titled "Going Concern".

Our ability to continue operations after our current cash resources are exhausted depends on our ability to obtain additional financing or to achieve profitable operations, as to which no assurances can be given. Cash requirements may vary materially from those now planned because of changes in direction of our research and development programs, competitive and technical advances, patent developments, regulatory changes, or other developments. If adequate additional funds are not available when required, or if we are unsuccessful in entering into partnership agreements for further development of our pipeline, management may need to curtail our development efforts and planned operations to conserve cash.

Until such time, if ever, as we can generate substantial product revenues to support our business and corporate strategy, we expect to finance our cash needs through a combination of public and private equity offerings, debt financings, at-the-market offerings, government or private party grants, collaborations, strategic alliances, and licensing arrangements. As of December 31, 2024, we did not maintain any lines of credit or equity capital committed for funding with the exception of the 2024 ATM Program.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends. If we raise funds through collaborations, or other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. We may not be able to obtain additional funds through equity or debt financings when needed on favorable terms or at all, including as a result of rising interest rates, liquidity concerns at, and failures of, banks and other financial institutions, volatility in the capital markets and

related market uncertainty. Further, if we are unable to obtain additional funding to support our current or proposed activities and operations, we may not be able to continue our operations as currently anticipated, which may require us to suspend or terminate any ongoing development activities, modify our business plan, curtail various aspects of our operations, cease operations, or seek relief under applicable bankruptcy laws.

Cash Flows

The following table summarizes our cash flows for the periods presented:

	Year Ended December 31,	
	2024	2023
Cash used in operating activities	$ (18,873,827)	$ (18,865,873)
Cash provided by investing activities	4,977,507	10,222,667
Cash provided by financing activities	13,012,076	12,641,343
Effect of exchange rate changes	(527,168)	488,404
Net (decrease)/increase in cash, cash equivalents and restricted cash	$ (1,411,412)	$ 4,486,541

Cash Flows from Operating Activities

During the years ended December 31, 2024 and 2023, we used $18.9 million of cash in operating activities, primarily to fund our operations related to the development of our pipeline and product candidates as well as related general and administrative support activities.

Cash Flows from Investing Activities

During the year ended December 31, 2024, net cash provided by investing activities was $5.0 million, primarily due to the maturity of marketable securities.

During the year ended December 31, 2023, net cash provided by investing activities was $10.2 million, primarily due to the maturity of marketable securities for $12.2 million partially offset by the purchase of marketable securities for $2.0 million.

Cash Flows from Financing Activities

During the year ended December 31, 2024, cash provided by financing activities was $13.0 million primarily related to the following: $10.3 million provided by net proceeds from the issuance of shares and warrants in the public offering, $3.0 million provided by net proceeds from issurance of shares in the ATM offering, $0.2 million provided by the exercise of stock options and public warrants, partially offset by $0.3 million payment of offering costs and $0.1 million repayment of long-term debt.

During the year ended December 31, 2023, cash provided by financing activities was $12.6 million mainly related to the net proceeds from the public offering and the concurrent private placement for $9.2 million, and the net proceeds related to the issurance of shares in the ATM offering of $3.5 million.

Funding Requirements

Our primary use of cash is to fund our operating expenses, which consist of research and development and general and administrative expenditures.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the scope, timing, progress and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates;

- the extent to which we enter into collaborations or other arrangements with additional third parties in order to further develop our product candidates;
- the extent to which we encounter increased costs as a result of global and macroeconomic conditions, including high interests rates, supply chain disruptions, fluctuating exchange rates, and increases in commodity, energy and fuel prices;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the costs and fees associated with the discovery, acquisition or in-license of additional product candidates or technologies;
- our ability to establish additional collaborations on favorable terms, if at all;
- the costs required to scale up our clinical, regulatory and manufacturing capabilities;
- the costs of manufacturing our product candidates for clinical trials and in preparation for marketing approval and commercialization;
- the costs of future commercialization activities, if any, including establishing sales, marketing, manufacturing and distribution capabilities, for any of our product candidates for which we receive marketing approval; and
- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval.

We will need additional funding to meet our operational needs and capital requirements for our preclinical studies and clinical trials, other research and development expenditures, and business development activities. Because of the numerous risks and uncertainties associated with the development of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our operations through a combination of equity offerings, including at-the-market offerings, debt financings, government or private party grants, collaborations, strategic alliances and licensing arrangements. We may not be able to obtain additional funds through equity or debt financings when needed on favorable terms or at all.

Critical Accounting Policies and Use of Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue, costs and expenses, and the disclosure of contingent assets and liabilities in our 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. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses, defined benefit pension liability, share-based compensation, recognition of research grants and the going concern assessment. Our actual results may differ from these estimates under different assumptions or conditions. During the year ended December 31, 2024, there were no material changes to our critical accounting policies. While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements included elsewhere in this Annual Report, we believe the following accounting policies are the most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Accrued expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses as of each balance sheet date. 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 the actual cost. We make estimates of our accrued expenses as of each balance sheet date based on facts and circumstances known to us at that time at the date of the preparation of the financial statements. There may be instances in which payments made to our vendors exceed the level of services provided, and result in a prepayment reported under other current assets, which are subsequently expensed in the Consolidated Statements of Operations when the related activity has been performed. To date, there have been no material differences between our estimates of accrued expenses reported at each balance sheet date and the amounts actually incurred.

Pension obligations

We operate defined benefit pension plans and defined contribution pension plans in accordance with local regulations and practices. These plans are funded by regular contributions made by the employer and the employees to a third-party. For defined benefit pension plans, the liability recognized in the balance sheets is the present value of the defined benefit obligation at the balance sheet date less the fair value of plan assets. The overfunded or underfunded status of the defined benefit plans is calculated as the difference between plan assets and the projected benefit obligations. Estimates are used in determining the assumptions incorporated in the calculation of the pension obligations, which is supported by input from independent actuaries. Actuarial gains and losses arising from experience adjustments and changes in actuarial assumptions are recognized in "Accumulated Other Comprehensive Income (Loss)" in the Consolidated Statements of Changes in Stockholders' Equity and are charged or credited to income over the employees' expected average remaining working lives. The measurement date used for our employee defined benefit plan is December 31st.

Share-based compensation

We recognize compensation costs related to share-based compensation granted to employees, consultants, and directors based on the estimated fair value of the awards as of the grant date. We estimate the grant date fair value and the resulting share-based compensation using the Black-Scholes option-pricing model for stock option awards. The grant date fair value of the stock option awards is recognized on a straight-line basis over the requisite service period, which is generally the vesting period of the respective awards. We estimate the fair value of stock options using the Black-Scholes option-pricing model, which requires assumptions, including volatility, the expected term of exercise, the risk free interest rate for a period that approximates the expected term of exercise, and our expected dividend yield. Certain assumptions used in our Black-Scholes option-pricing model represent management's best estimates and involve a number of assumptions and the application of management's judgment, as they are inherently subjective. We recognize expenses related to Restricted Stock Units (or RSUs) based on their fair market value, determined as the closing price on the Nasdaq of our common stock as of the grant date, on a straight-line basis over the requisite service period. For restricted stock units with market or performance based vesting conditions (or PRSUs), the fair value at grant date is calculated using an option-pricing model (Monte Carlo Simulation) or based on management's assessment of the likelihood of occurrence of the underlying performance.

Research grants

Under the terms of the research and development grants awarded, we are entitled to receive upfront payments or reimbursement of our allowable direct expenses. Contributions from research and development activities under the grants are recorded based on management's best estimate of the periods in which the related expenditures are incurred and activities performed and are classified in the Consolidated Statements of Operations as a reduction to research and development expenses.

Under the Australian government's Research and Development Tax Incentive ("R&DTI") program, we are also eligible to obtain certain research and development tax credits. The tax credits are available on the basis of specific criteria with which we must comply. The tax credits are administered through the local tax authority and can be realized regardless of whether we have generated taxable income in the respective jurisdictions. The tax credits are based on a percentage of eligible research and development activities under the program and are recorded based on management's best estimate of the periods in which the related expenditures are incurred and activities performed and

are classified in the Consolidated Statements of Operations as a reduction to research and development expenses when collectability is reasonably assured.

Going concern assessment

At each reporting period, the Company evaluates whether there are conditions or events that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the financial statements are issued. The assessment over going concern is based on management's most updated budget and forecast and does not take into consideration estimated future cash inflows that are not certain as of the date of preparation of the financial statements.

Jumpstart Our Business Startups ("JOBS") Act

We qualify as an "emerging growth company", as defined in the JOBS Act. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to public companies that are not emerging growth companies. These provisions include, but are not limited to: being permitted to report only two years of audited financial statements and only two years of related selected financial data and management's discussion and analysis of financial conditions and results of operations disclosure; an exemption from compliance with the auditor attestation requirement in the assessment of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act; reduced disclosure obligations regarding executive compensation arrangements in our periodic reports, registration statements and proxy statements; and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, the JOBS Act permits emerging growth companies to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. As a result, the information we provide might be different from the information that is available for other public companies. We cannot predict whether investors will find our common stock less attractive if we 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 the market price of our common stock may be more volatile.

We will remain an emerging growth company until the earliest of (i) the last day of our first fiscal year in which we have total annual gross revenue of $1.235 billion or more, (ii) December 31, 2026, (iii) the date on which we have issued more than $1.0 billion of non-convertible debt instruments during the previous three fiscal years or (iv) the date on which we are deemed a "large accelerated filer" under the rules of the SEC with at least $700 million of outstanding equity securities held by non-affiliates.

Recent Accounting Pronouncements

A description of recent accounting pronouncements that may potentially impact our financial position, results of operations or cash flows is disclosed in Note 2 to our audited financial statements included elsewhere in this Annual Report.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISKS

Under SEC rules and regulations, because we are considered to be a "smaller reporting company", we are not required to provide the information required by this item in this Annual Report.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

INDEX TO FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Gain Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Gain Therapeutics, Inc. (the "Company") as of December 31, 2024 and 2023, the related consolidated statements of operations, comprehensive loss, changes in stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2024, in conformity with U.S. generally accepted accounting principles.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations since inception and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (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 consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated 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 consolidated 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 consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young AG

We have served as the Company's auditor since 2020.
Lugano, Switzerland
March 27, 2025

Gain Therapeutics, Inc
Consolidated Balance Sheets

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 10,385,863	$ 11,794,949
Marketable securities	—	4,999,704
Tax credits	271,079	242,577
Prepaid expenses and other current assets	945,536	741,638
Total current assets	11,602,478	17,778,868
Noncurrent assets:		
Property and equipment, net	103,619	125,962
Internal-use software	134,268	193,375
Operating lease right-of-use assets	219,715	459,215
Restricted cash	31,695	34,021
Long-term deposits and other noncurrent assets	32,109	17,890
Total noncurrent assets	521,406	830,463
Total assets	$ 12,123,884	$ 18,609,331
Liabilities and stockholder's equity		
Current liabilities:		
Accounts payable	$ 946,259	$ 1,318,965
Operating lease liability - current	160,913	229,693
Other current liabilities	2,441,761	2,160,366
Deferred grant income - current	252,211	1,122,138
Loans - current	110,177	118,797
Total current liabilities	3,911,321	4,949,959
Noncurrent liabilities:		
Defined benefit pension plan	443,623	307,454
Operating lease liability - noncurrent	53,598	229,855
Deferred grant income - noncurrent	47,441	94,786
Loans - noncurrent	328,327	449,053
Total noncurrent liabilities	872,989	1,081,148
Total liabilities	$ 4,784,310	$ 6,031,107
Stockholders' equity		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized; nil shares issued and outstanding as of December 31, 2024 and 2023	$ —	$ —
Common stock, $0.0001 par value: 50,000,000 shares authorized; 27,132,588 and 16,206,680 shares issued and outstanding as of December 31, 2024 and 2023, respectively	2,713	1,621
Additional paid-in capital	88,779,318	73,113,079
Accumulated other comprehensive (loss) income	(247,549)	247,241
Accumulated deficit	(60,783,717)	(38,516,197)
Loss of the period	(20,411,191)	(22,267,520)
Total stockholders' equity	7,339,574	12,578,224
Total liabilities and stockholders' equity	$ 12,123,884	$ 18,609,331

Gain Therapeutics, Inc
Consolidated Statements of Operations

	Year Ended December 31,	
	2024	2023
Revenues:		
Collaboration revenues	$ —	$ 55,180
Other income	—	—
Total revenues	—	55,180
Operating expenses:		
Research and development	(10,791,058)	(11,520,613)
General and administrative	(9,559,534)	(10,787,700)
Total operating expenses	(20,350,592)	(22,308,313)
Loss from operations	(20,350,592)	(22,253,133)
Other income (expense):		
Interest income, net	357,096	494,234
Foreign exchange gain (loss), net	119,120	(429,346)
Loss before income tax	(19,874,376)	(22,188,245)
Income tax	(536,815)	(79,275)
Net loss	$ (20,411,191)	$ (22,267,520)
Net loss per shares:		
Net loss per share attributable to common stockholders - basic and diluted	$ (0.89)	$ (1.71)
Weighted average common stock - basic and diluted	22,881,415	13,011,361

Gain Therapeutics, Inc
Consolidated Statements of Comprehensive Loss

	Year Ended December 31,	
	2024	2023
Net loss	$ (20,411,191)	$ (22,267,520)
Components of other comprehensive (loss) income		
Unrealized gain on available-for-sale marketable securities	4,974	89,304
Changes in defined benefit pension plan obligation, net of tax	(132,524)	(127,601)
Foreign currency translation	(367,240)	249,911
Other comprehensive (loss) income, net of tax	(494,790)	211,614
Comprehensive loss	$ (20,905,981)	$ (22,055,906)

Gain Therapeutics, Inc
Consolidated Statements of Changes in Stockholders' Equity

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total
	Shares	Amounts				
Balance as of December 31, 2022	**11,883,368**	**$ 1,189**	**$ 57,358,895**	**$ 35,627**	**$ (38,516,197)**	**$ 18,879,514**
Stock-based compensation (Note 15)	171,751	16	3,305,056	—	—	3,305,072
Changes in defined benefit pension plan obligation (Note 11)	—	—	—	(127,601)	—	(127,601)
Foreign currency translation	—	—	—	249,911	—	249,911
Net unrealized gain on available-for-sale securities (Note 5)	—	—	—	89,304	—	89,304
Issuance of shares in at-the-market (ATM) offering (Note 14)	862,535	86	3,544,790	—	—	3,544,876
Issuance of shares and warrants in public offering and private placement (Note 14)	3,289,026	330	8,904,338	—	—	8,904,668
Net loss	—	—	—	—	(22,267,520)	(22,267,520)
Balance as of December 31, 2023	**16,206,680**	**$ 1,621**	**$ 73,113,079**	**$ 247,241**	**$ (60,783,717)**	**$ 12,578,224**
Stock-based compensation (Note 15)	77,777	7	2,341,213	—	—	2,341,220
Changes in defined benefit pension plan obligation (Note 11)	—	—	—	(132,524)	—	(132,524)
Foreign currency translation	—	—	—	(367,240)	—	(367,240)
Net unrealized gain on available-for-sale securities (Note 5)	—	—	—	4,974	—	4,974
Issuance of shares in ATM offering (Note 14)	1,597,128	159	2,950,974	—	—	2,951,133
Issuance of shares and warrants in public offering and private placement (Note 14)	9,251,003	926	10,374,052	—	—	10,374,978
Net loss	—	—	—	—	(20,411,191)	(20,411,191)
Balance as of December 31, 2024	**27,132,588**	**$ 2,713**	**$ 88,779,318**	**$ (247,549)**	**$ (81,194,908)**	**$ 7,339,574**

Gain Therapeutics, Inc
Consolidated Statements of Cash Flows

	Year Ended December 31,	
	2024	2023
Operating activities:		
Net loss	$ (20,411,191)	$ (22,267,520)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	84,071	83,579
Stock based compensation expense	2,390,159	3,259,026
Other non cash items	143,523	(383,188)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(356,650)	54,378
Long term deposit and other noncurrent assets	(21,000)	(10,057)
Accounts payable and other current liabilities	274,889	(693,698)
Defined benefit pension plan	(122,915)	6,313
Deferred grant income	(854,713)	1,085,294
Total changes in operating assets and liabilities	(1,080,389)	442,230
Cash used in operating activities	(18,873,827)	(18,865,873)
Cash flows from investing activities:		
Purchase of property and equipment and internal-use of software	(22,493)	(15,358)
Purchases of marketable securities	—	(1,956,350)
Maturities of marketable securities	5,000,000	12,194,375
Cash provided by investing activities	4,977,507	10,222,667
Cash flow from financing activities:		
Net proceeds from issuance of shares in ATM offering (Note 14)	2,951,133	3,544,876
Net proceeds from issuance of shares and warrants in public offering (Note 14)	10,261,178	9,185,534
Net proceeds from the exercise of warrants (Note 14)	113,800	—
Net proceeds from the exercise of stock options (Note 15)	57,711	—
Payments of offering costs (Note 14)	(280,867)	—
Payments of current portion of long-term debt (Note 12)	(90,879)	(89,067)
Cash provided by financing activities	13,012,076	12,641,343
Effect of exchange rate changes	(527,168)	488,404
Net (decrease)/increase in cash, cash equivalents and restricted cash	(1,411,412)	4,486,541
Cash, cash equivalents and restricted cash at beginning of period	11,828,970	7,342,429
Cash, cash equivalents and restricted cash at end of period	$ 10,417,558	$ 11,828,970
Supplemental Data:		
Income taxes paid	$ 86,309	$ 134,962

Notes to the Consolidated Financial Statements

1. Nature of the Business and Basis of Presentation

Operations and Business

Gain Therapeutics, Inc. (and together with its subsidiary, the "Company"), was incorporated under the laws of the state of Delaware (U.S.) on June 26, 2020. Gain Therapeutics has been a publicly traded company since the Initial Public Offering ("IPO") completed in March of 2021. The shares trade on the Nasdaq Global Market under the ticker symbol "GANX".

The Company is a biotechnology company developing novel small molecule therapeutics to treat diseases across several therapeutic areas, including, central nervous system ("CNS") disorders, lysosomal storage disorders ("LSDs"), metabolic disorders, and other diseases that can be targeted through protein degradation, such as oncology.

The Company's clinical stage product candidate, GT-02287, is being developed for the treatment of Parkinson's disease with and without GBA1 mutations. The Company generated an extensive preclinical data package providing evidence of the mechanism of action, in vivo pharmacology, and safety of GT-02287. In preclinical models of GBA1 Parkinson's disease, GT-02287 has been shown to restore glucocerebrosidase, or GCase, function in the lysosome, reduce toxic lipid substrates and toxic forms of alpha-synuclein, reduce endoplasmic reticulum stress, improve mitochondrial health and overall survival of dopaminergic neurons, increasing dopamine levels, restoring locomotor and cognitive function, and reducing plasma-based neurodegeneration maker, neurofilament light chain (NfL), back to the level of control animals.

The Company uses the Magellan™ drug discovery platform to identify novel allosteric sites and small molecules for all its pipeline programs. The Company plans to continue to advance its existing research programs and initiate additional programs targeting allosteric binding sites identified with the Magellan™ platform in various therapeutic areas through academic partnerships, co-development, and licensing arrangements.

Risks and Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, risks associated with completion and success of preclinical studies and clinical testing, dependence on key personnel, protection of proprietary technology, compliance with applicable governmental regulations, development by competitors of new technological innovations, protection of proprietary technology and the ability to secure additional capital to fund operations. Drug candidates currently under development will require significant additional research and development efforts, including preclinical and clinical testing and prior to regulatory approval and commercialization. These efforts require significant amounts of additional capital, adequate personnel, infrastructure and extensive compliance-reporting capabilities. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

Basis of Presentation

The accompanying audited annual consolidated financial statements (the "annual financial statements") reflect the accounts of Gain Therapeutics, Inc., Gain Therapeutics Australia PTY LTD, GT Gain Therapeutics SA and its wholly owned branch, Gain Therapeutics Sucursal en España. All intercompany transactions and balances have been eliminated in the preparation of the consolidated financial statements. The annual financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

The annual financial statements have been prepared on the same basis as applied for the audited annual consolidated financial statements as of and for the year ended December 31, 2023, and, in the opinion of management, reflect all adjustments, consisting of normal recurring adjustments, necessary for the fair presentation of the Company's financial position as of December 31, 2024, the results of its operations and its statements of stockholders' equity and its statements of cash flows for the years ended December 31, 2024 and 2023. All amounts in the

consolidated financial statements are expressed in U.S. dollars and disclosed within these explanatory notes in U.S. dollars.

The results for the years ended December 31, 2024 and 2023 are not necessarily indicative of the results to be expected for any future year or period. These annual financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2023, and the notes thereto, which are included in the Annual Report.

These accompanying annual financial statements reflect the application of significant accounting policies as described below and elsewhere in these notes to the audited consolidated financial statements. As of December 31, 2024, the Company's significant accounting policies and estimates, which are detailed herein, have not changed.

Going Concern

At each reporting period, the Company evaluates whether there are relevant conditions or events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the financial statements are issued.

The Company has incurred recurring losses and negative cash flows from operations since its inception and has primarily funded these losses through the completion of its IPO in March 2021, other equity financings and research grants. The Company anticipates incurring additional losses until such time, if ever, that it can generate significant sales of its product candidates currently in development. Substantial additional capital will be needed by the Company to fund its operations and to develop its product candidates.

The Company's activities have consisted primarily of performing research and conducting preclinical and clinical studies, organizing and staffing the Company, expanding its operations, securing financing, developing and securing its in-licensed technology. The Company faces risks associated with early-stage biotechnology companies whose product candidates are in development. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing, establishing manufacturing capacity and obtaining regulatory approval prior to commercialization. These efforts require significant amounts of additional capital for the Company to complete its research and development activities, achieve its research and development objectives, defend its intellectual property rights, and recruit and retain skilled personnel, and key members of management. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from product sales.

In accordance with ASU No. 2014-15, "Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern", the Company has evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the financial statements are issued. The Company assessed that its existing cash and cash equivalents of $10.4 million as of December 31, 2024 will not be sufficient to fund its estimated operating and capital expenditures for a period of at least 12 months from the date these financial statements are issued. Because of the current liquidity situation and lack of expected revenues in the foreseeable future, substantial doubt exists about its ability to continue as going concern. The Company will need to obtain additional capital and/or other funding in order to continue operations beyond the third quarter of 2025.

Management plans to raise additional capital primarily through private and/or public equity financings and/or convertible debt financings. As an additional action, management is currently reviewing the cost structure throughout the organization, looking for opportunities to optimize expenditures and create efficiencies with the objective of improving the Company's overall cash burn rate, optimizing the research and development expenses and reducing general and administrative expenses. Furthermore, management is actively seeking opportunities for strategic collaborations, licensing agreements and grant fundings, among other strategic opportunities.

The Company may not be successful in its efforts to raise additional funds or achieve profitable operations. The Company continues to explore potential opportunities and alternatives to obtain the additional resources that will be necessary to support its ongoing operations beyond the third quarter of 2025, including raising additional capital through either private or public equity, debt financing, or additional program collaborations.

If the Company is unable to obtain additional funding to support its current or proposed activities and operations, it may not be able to continue its operations as currently anticipated, which may require it to suspend or terminate any ongoing development activities, modify its business plan, curtail various aspects of its operations, cease operations, or seek relief under applicable bankruptcy laws. In such event, the Company's stockholders may lose a substantial portion or even all of their investment.

Because of the actions that management is taking to secure future financial resources, the accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. The financial statements do not include any adjustments that might be necessary if the Company is unable to continue as going concern.

Segment Information

Operating segments are defined as components of an entity for which separate financial information is available and that is regularly reviewed by the Chief Operating Decision Maker ("CODM") in deciding how to allocate resources and in assessing performance. The Company's Chief Executive Officer is the Company's CODM. The CODM reviews financial information presented on a consolidated basis for purposes of making operating decisions, allocating resources, and evaluating financial performance. As such, the Company has determined that it operates as one operating segment, which is research and development in the pharmaceutical sector with a focus on developing novel therapeutics to treat diseases caused by protein misfolding. The Company has concluded that consolidated net income (loss) is the measure of segment profitability. The CODM assesses performance for the Company, monitors budget versus actual results, and determines how to allocate resources based on consolidated net income (loss) as reported in the Consolidated Statements of Operations. There are no other expense categories regularly provided to the CODM that are not already included in the primary financial statements herein.

The Company has operations in four (4) geographic locations: Switzerland, Spain, the United States, and Australia. Product candidates currently under development require significant additional research and development efforts, including extensive preclinical and clinical testing, establishing manufacturing capacity and obtaining regulatory approval prior to commercialization, as such no revenue has been generated and therefore no additional disclosures of revenue information from products and services and information on major customers are required. The following table details the Company's long-lived assets by geographic location, with the exception of Australia where there are no long-lived assets:

	December 31, 2024	December 31, 2023
Long-lived assets by geographic location		
Switzerland	$ 282,185	$ 445,507
Spain	164,847	274,855
United States	10,570	58,190
Total	$ 457,602	$ 778,552

2. Summary of Significant Accounting Policies

Foreign Currency Transactions

The Company is incorporated in the United States of America and has operations in Switzerland, Spain and Australia. The Company's reporting currency is U.S. dollars ("USD"). The functional currencies of the Company's operations are the local currencies (USD in the United States, Swiss franc in Switzerland, euro in Spain and Australian dollar in Australia). Assets and liabilities reported in the Consolidated Balance Sheets are translated into USD (the currency in which these financial statements are presented) at the exchange rates applicable at the balance sheet dates and for the Consolidated Statements of Operations at the average exchange rates for the periods presented. Items representing the share capital and additional paid-in capital are presented at historical exchange rates. Adjustments resulting from the translation of the financial statements of the Company's foreign operations into USD are excluded from the determination of net income and are recorded in accumulated other comprehensive income/(loss), a separate component of shareholders' equity. The Company has not utilized any foreign currency hedging strategies to mitigate the effect of its foreign currency exposure. As of December 31, 2024 and December 31, 2023, accumulated currency translation adjustments recorded in accumulated other comprehensive loss amounted to $41,247 and $408,487, respectively.

Use of Estimates

The preparation of the Company's consolidated financial statements in conformity with U.S. GAAP requires management to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenue and expenses during the reporting periods. On an ongoing basis, the Company evaluates its estimates, judgments and assumptions including those related to going concern assessment, recognition of accrued expenses, defined benefit pension liability, share-based compensation, and recognition of research grants. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable by management under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates. Changes in estimates are recorded in the period in which they become known. To the extent that material differences arise between the estimates and actual results, the Company's future results of operations will be affected.

Cash and Cash Equivalents

The Company classifies cash on hand and held at banks, and all highly liquid investments in money markets, certificates of deposit, time deposits, and other short-term liquid securities with original maturities of less than 90 days, as cash and cash equivalents.

Marketable Securities

The Company classifies marketable securities as held-to-maturity or available-for-sale at the time these instruments are purchased, based on the requirements of ASC 320, "Investments – Debt Securities" ("ASC 320").

Marketable securities are classified as available-for-sale since the Company does not have the positive intent and the capacity to hold the marketable securities until the maturity date. Available-for-sale marketable securities are carried out at fair value with the unrealized gain/(loss) excluded from the computation of the earnings of the period and accounted for in other comprehensive income/(loss). The accretion of discounts (or amortization of premiums) is accounted for in the Company's Consolidated Statements of Operations as interest income or (expense).

Marketable securities are classified in the Company's Consolidated Balance Sheets based on their maturities and the Company's reasonable expectations with regard to those securities. Marketable securities with a maturity date within 12 months from the reporting date are classified as "Current assets." Marketable securities with a maturity date over 12 months from reporting date are classified as "Noncurrent assets."

Concentrations of Credit Risk

The Company has no significant off-balance sheet risk, such as foreign exchange contracts, option contracts, or other foreign hedging arrangements. Financial instruments that may expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents which are deposited in accredited financial institutions in excess of federally insured limits. The Company deposits its cash and cash equivalents in financial institutions that it believes have high credit quality and have 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.

Deferred Issuance Costs

The Company capitalizes certain legal, professional accounting, and other third-party fees that are directly associated with in-process equity financings as deferred issuance costs until such equity transactions are consummated. Subsequently, these costs are recorded as a reduction of the proceeds generated as a result of the offering. Should the planned equity financing be abandoned, the deferred issuance costs will be expensed immediately as a charge to operating expenses in the Consolidated Statements of Operations.

Property and Equipment

Property and equipment are stated at cost, including any accessory and direct costs that are necessary to make the assets fit for use, and adjusted by the corresponding accumulated depreciation. The depreciation expenses are recorded using the straight-line method in the Consolidated Statements of Operations and have been calculated by taking into consideration the use, purpose and financial-technical duration of the assets, on the basis of their estimated useful economic lives. The Company believes the above criteria to be represented by the following depreciation rates:

-	Equipment & furniture	12.5 %
-	Electronic office equipment	20 %
-	Leasehold improvements	based on the terms of the lease
-	Laboratory equipment	15 %

Ordinary maintenance costs are entirely attributed to the Consolidated Statements of Operations in the year in which they are incurred. Extraordinary maintenance costs, the purpose of which is to extend the useful economic life of the asset, to technologically upgrade it and/or to increase its productivity or safety for the purposes of the economic productivity of the Company, are attributed to the asset to which they refer and depreciated on the basis of its estimated useful economic lives. Amortization of leasehold improvements is computed using the straight-line method based upon the terms of the applicable lease or estimated useful life of the improvements, whichever is lower.

Capitalized Software Development Costs

The Company capitalizes the costs of software obtained for internal use in accordance with ASC 350-40, "Internal-Use Software". Capitalized software development costs consist of costs incurred during the development stage and include purchased software licenses, implementation costs, consulting costs, and payroll-related costs for projects that qualify for capitalization. All other costs, primarily related to maintenance and minor software fixes, are expensed as incurred.

Internal-use software, net consisted of the following:

	December 31, 2024	December 31, 2023
Internal-use software	$ 264,062	$ 284,637
Less: accumulated amortization	(129,794)	(91,262)
Internal-use software, net	$ 134,268	$ 193,375

The Company amortizes the capitalized software development costs on a straight-line basis over the estimated useful life of the software, which is generally six years, beginning when the asset is substantially ready for use. The amortization of capitalized software development costs is reflected in general and administrative expenses. Amortization expense for the years ended December 31, 2024 and December 31, 2023 was $47 thousand and $46 thousand, respectively.

Impairment of Long-lived Assets

In accordance with ASC 360-10-20, "Property, Plant and Equipment," the Company performs an impairment test whenever events or circumstances indicate that the carrying value of long-lived assets with finite lives may be impaired. Impairment is measured by comparing the carrying value of the long-lived assets to the estimated undiscounted pre-tax cash flows expected to result from the use of such assets and their ultimate disposition. In circumstances where impairment is determined to exist, the Company will write down the asset to its fair value based on the present value of estimated cash flows. No impairments have been identified by management as of and for any periods presented.

Patents

Patent-related costs refer to legal fees incurred in connection with filing and prosecuting patent applications and are expensed as incurred due to uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Leases

The Company determines if an arrangement contains a lease at inception based on whether or not the Company has the right to control the asset during the contract period and other facts and circumstances, as per ASC 842, "Leases." Operating lease right-of-use ("ROU") assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the obligation to make lease payments arising from the lease, both of which are recognized based on the present value of the future minimum lease payments over the lease term at the commencement date using the Company's incremental borrowing rate applicable to the lease. The collateralized incremental borrowing rate is based on the information available at the lease commencement date. The Company is typically required to make fixed minimum rent payments and is often required, by the lease, to pay for certain other costs including real estate taxes, insurance, common area maintenance and/or certain other costs, which may be fixed or variable, depending upon the terms of the respective lease agreement. To the extent these payments are fixed, the Company has included them in calculating the lease ROU assets and lease liabilities. Variable lease payments primarily include payments for non-lease components, such as maintenance or utilities. Leases with a term of 12 months or less at inception are expensed on a straight-line basis over the lease term in the Consolidated Statements of Operations. The Company determines the lease term by assuming the exercise of renewal options that are reasonably certain.

Accounts Payable

Accounts payable are reported at their nominal amounts due to their short-term maturities. Trade accounts payable are recorded net of trade discounts; cash discounts are recorded at the time of payment.

Payables for Social Security Charges

Social security charges are reported in compliance with rules and laws applicable in the countries where the Company's employees work. Charges are accrued in accordance with the policies stipulated and in connection with salaries due for the period.

Accrued Expenses

As part of the process of preparing the Company's consolidated financial statements, the Company is required to estimate its accrued expenses as of each balance sheet date. This process involves reviewing open contracts and purchase orders, communicating with the Company personnel to identify services that have been performed on its behalf and estimating the level of service performed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of the actual cost. The Company makes estimates of its accrued expenses as of each balance sheet date based on facts and circumstances known at the time of the preparation of its consolidated financial statements. There may be instances in which payments made to the Company's vendors exceed the level of services provided, and result in a prepayment reported under Other Current Assets, which is subsequently expensed in the Consolidated Statement of Operations when the related activity has been performed. To date, there have been no material differences between the Company's estimates of accrued expenses reported at each balance sheet date and the amounts actually incurred.

Pension Obligations

The Company operates defined benefit pension plan and defined contribution pension plans in accordance with local regulations and practices in the countries in which the Company operates. These plans are funded by regular contributions made by the Company and its employees. For the defined benefit pension plan, the liability recognized in the Consolidated Balance Sheets is the present value of the defined benefit obligation at the balance sheet date less the fair value of plan assets. The overfunded or underfunded status of the defined benefit plan is calculated as the difference between plan assets and the projected benefit obligations. Estimates are used in determining the assumptions incorporated in the calculation of the pension obligations, which is supported by input from independent actuaries. Actuarial gains and losses arising from experience adjustments and changes in actuarial assumptions are recognized in the Consolidated Statements of Changes in Stockholders' Equity under accumulated other comprehensive income (loss), and are charged or credited to income over the employees' expected average remaining service period using the corridor amortization method. The measurement date used for the Company's employees defined benefit plan is December 31.

For defined contribution pension plans, the Company pays contributions to publicly or privately administered pension insurance plans on a mandatory, contractual or voluntary basis. The Company has no further payment obligations once the contributions have been paid. The contributions are recognized as employee benefit expense when they are due.

Stock-based Compensation and Warrants

The Company issues stock-based compensation with service-based and performance-based vesting conditions. The Company applies the fair value method of measuring equity-based compensation and warrants, which requires an entity to measure the cost of services received in exchange for an award of equity instruments based on the grant-date fair value of the award. The Company recognizes the corresponding expense in the statement of operations over the period the participants are required to render service. Forfeitures are recognized as they occur.

The fair value of each stock option award is estimated as of the grant date using the Black-Scholes option pricing model. The Company determines the volatility and the expected term of exercise for awards granted based on the actual volatility of its share price traded on the Nasdaq and the best estimate of the timing of the exercise by the beneficiaries as of the grant date. 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. The Company has not paid, and does not anticipate paying, cash dividends on its common stock; therefore, the expected dividend yield is assumed to be nil.

The Company recognizes expenses related to restricted stock units (RSUs) based on their fair market value, determined as the closing price on the Nasdaq of the Company's common stock as of the grant date, on a straight-line basis over the requisite service period. For restricted stock units with performance-based vesting conditions (PRSUs), the fair value at grant date is calculated based on management's assessment of the likelihood of occurrence of the underlying performance.

The Black-Scholes option pricing model is also used for the warrants issued, using consistent inputs and methodology to quantify such inputs, as described above in relation to equity-based compensation.

The assumptions used in calculating the fair value of share-based awards and warrants represent management's best estimates and involve inherent uncertainties and the application of management's judgment.

Revenue Recognition

When the Company derives income from its collaboration and licensing agreements, it recognizes revenue in accordance with ASC 606, "Revenues from Contracts with Customers" ("ASC 606") and ASC 808, "Collaborative Arrangements". The terms of these arrangements typically include payment from third-party customers of one or more of the following: non-refundable initiation fee, reimbursement of development costs, future development and regulatory milestone payments, and royalties on net sales of the licensed product.

In determining the appropriate amount of revenue to be recognized as the Company fulfills its obligations, the Company applies the five-step model of ASC 606: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) it satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods and services it transfers to the customer. If a contract is determined to be within the scope of ASC 606 at inception, the Company assesses the goods or services promised within such contract, determines which of those goods and services 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.

Costs and revenues associated with collaborative arrangements are reported in the Consolidated Statements of Operations on a gross basis when the counterpart is identified as being a customer, when the performance obligations incurred and rendered to fulfill the agreements are deemed to be in the ordinary course of the Company's business, or when there is an expectation that the collaborative arrangement will result in a future constant flow of revenues in the form of sales of products, royalties, or licenses.

Research Grants

Under the terms of the research and development grants awarded, the Company receives upfront payments or is entitled to receive reimbursement of its allowable direct expenses and payroll costs. Contributions from research and development activities under the grants are recorded when there is reasonable assurance of collection and based on management's best estimate of the periods in which the related expenditures are incurred, and activities performed and are classified in the Consolidated Statements of Operations as a reduction to research and development expenses. Grants received in advance of the specific research and development costs to which they relate are deferred and recognized in the Consolidated Balance Sheets as deferred grant income.

The Company is also eligible to obtain certain research and development tax credits. The tax credits are available on the basis of specific criteria with which the Company must comply. The tax credits are administered through the local tax authority and can be realized regardless of whether the Company has generated taxable income in the respective jurisdictions. The Company accounts for the tax credits through offsets to the related research and development as it incurs costs eligible for reimbursement when collectability is reasonably assured and when the applicable conditions under the tax incentive program have been met.

Research and Development Expenses

The Company expenses all costs incurred in performing research and development activities. Research and development expenses include salaries and other related costs, materials and supplies, preclinical expenses, manufacturing expenses, contract services, and other third-party expenses.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, benefits, and other related costs, for personnel and consultants in the Company's executive, administrative, and finance functions. General and administrative expenses also include professional fees for legal, finance, accounting, intellectual property, auditing, tax and consulting services, travel expenses and facility-related expenses, which include allocated expenses for rent and maintenance of facilities and other operating costs not otherwise included in research and development expenses.

Income Taxes

The Company accounts for income taxes under the liability method. Under this method deferred income tax liabilities and assets are determined based on the difference between the financial statements carrying amounts of assets and liabilities and the related tax basis using enacted tax rates in effect in the years in which the associated deferred taxes are expected to reverse. A valuation allowance is recorded if it is "more likely than not" that a portion or all of a deferred tax asset will not be realized.

As of each reporting date, the Company considers existing evidence, both positive and negative, that could impact its view with regard to future realization of deferred tax assets. In consideration of the start-up status of the Company, a full valuation allowance has been established to offset the deferred tax assets, as the related realization is currently uncertain. In the future, should management conclude that it is more likely than not that the deferred tax assets are partially or fully realizable, the valuation allowance will be reduced to the extent of such expected realization, and the corresponding amount will be recognized as income tax benefit in the Company's Consolidated Statements of Operations.

Fair Value Measurement

The Company defines fair value as the price that would be received from selling an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. The Company applies the following fair value hierarchy, which prioritizes the inputs used to measure fair value into three levels based on their observability in the market and degree of judgment involved:

- Level 1 – Quoted prices in active markets for identical assets or liabilities.
- Level 2 – Observable inputs other than quoted prices in active markets for identical assets and liabilities, quoted prices for identical or similar assets or liabilities in inactive markets, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.

- Level 3 – Inputs that are generally unobservable and typically reflect management's estimates of assumptions that market participants would use in pricing the asset or liability.

In determining fair value, the Company utilizes valuation techniques that maximize the use of observable inputs and minimize the use of unobservable inputs to the extent possible and consider counterparty credit risk in their assessment of fair value.

Comprehensive Income / (Loss)

Comprehensive income / (loss) is composed of net income / (loss) and certain changes in stockholder's equity that are excluded from the net income / (loss), primarily foreign currency translation adjustments, changes in defined benefit obligation, and unrealized gains / (losses) on available-for-sale securities.

Net Loss per Share

Basic net loss per share is computed by dividing the reported net loss by the weighted average number of shares of common stock outstanding during the period and shares issuable for little or no cash consideration upon resolution of any applicable contingency. The Company gives consideration to all potentially dilutive impacts, except where the effect of including such securities would be anti-dilutive. As of December 31, 2024 and December 31, 2023, common stock equivalents consisted of stock options, RSUs, PRSUs and warrants. Because the Company has reported net losses since inception, these potential impacts would be anti-dilutive, and therefore common stock equivalents have been excluded from the computation, resulting in basic and diluted net loss per share being the same for all periods presented.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies that the Company adopts as of the specified effective date.

Recently issued accounting pronouncements adopted

In November 2023, the FASB issued ASU No. 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*. This ASU primarily requires incremental disclosures of disaggregated expense information about a Company's reportable segments. The Company adopted this ASU for the year-end December 31, 2024, and applied it retrospectively to all prior periods presented (see Note 1).

Recently issued accounting pronouncements not yet adopted

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures* . This ASU primarily requires disaggregated information about a Company's effective tax rate reconciliation as well as information on income taxes paid. The ASU is effective on a prospective basis for annual periods beginning after December 15, 2024. This ASU will likely result in the required additional disclosures being included in the Company's consolidated financial statements, once adopted. As this ASU relates to disclosures only, there will be no impact to the Company's consolidated results of operations and financial condition.

In November 2024, the FASB issued ASU No. 2024-03, *Disaggregation of Income Statement Expenses (Subtopic 220-40)*. The ASU requires new disclosures providing further detail of a company's income statement expense line items. The ASU is effective for annual periods beginning after December 15, 2026, and interim periods within fiscal years beginning after December 15, 2027. Adoption of this ASU can either be applied prospectively to consolidated financial statements issued for reporting periods after the effective date of this ASU or retrospectively to any or all prior periods presented in the consolidated financial statements. This ASU will likely result in the required additional disclosures being included in the Company's consolidated financial statements, once adopted. As this ASU relates to disclosures only, there will be no impact to the Company's consolidated results of operations and financial condition.

3. Research Grants

During the course of its business, the Company applies for research grants with public or private organization to funds its research projects. Under the terms of these grants, the Company receives an upfront payment or is entitled to receive reimbursement of its allowable direct research expenses.

In March 2023, the Company's wholly owned subsidiary, GT Gain Therapeutics SA, announced that Eurostars and Innosuisse awarded a grant in the aggregated amount of $1.3 million to a consortium led by Gain Therapeutics that also includes the Institute for Research in Biomedicine, Newcells Biotech and the University of Helsinki. The grant is intended to support the development of our alpha-1 antitrypsin deficiency program. The portion of the grant that was allocated to the Company was $0.45 million.

In May 2023, the Company's wholly owned subsidiary, GT Gain Therapetuics SA, announced that Innosuisse awarded the Company a grant, under the Swiss Accelerator program, in the amount of $2.8 million to support the further development activities of Gain Therapeutics' lead program GBA1 Parkinson's disease. In December 2024, the parties to the grant entered into an amendment to the funding agreement whereby the grant amount was amended to approximately $2.0 million due to changes in project scope. There is no impact to the Consolidated Statements of Operations or Consolidated Balance Sheets due to this amendment.

In connection with the grants announced in March 2023 and May 2023 the Company recorded a reduction to research and development expenses of $0.9 million and $0.6 million and reports deferred grant income of $0.3 million and $1.2 million during the years ended December 31, 2024 and December 31, 2023, respectively.

In the second quarter of fiscal year 2023, the Company's wholly owned subsidiary, Gain Therapeutics Australia started the Phase 1 Clinical Trial for its lead program in Parkinson's disease in Australia. The Australian government sponsors the Research and Development Tax Incentive ("R&DTI") program which offers a tax credit for companies conducting eligible research and development activities. The R&DTI program provides for a cash refund based on a percentage of eligible research and development activities undertaken in Australia by Gain Therapeutics Australia. The Company recorded a reduction to research and development expenses of $1.3 million during the year ended December 31, 2024 related to the R&DTI program. The corresponding receivable was recorded within Other Current Assets (see Note 6).

4. Cash, Cash Equivalents and Restricted Cash

The Company considers all short-term, highly liquid investments, with an original maturity of three months or less, to be cash equivalents. The Company's cash and cash equivalents include short-term highly liquid investments which are readily convertible into cash and relate to money market securities. The Company's institutional money market accounts permit daily redemption, and the fair values of these investments are based upon the quoted prices in active markets provided by the holding financial institutions, which are considered Level 1 inputs in the fair value hierarchy. Given their short-term maturities and the underlying value being mainly represented by cash equivalents, their face value amount approximates the related fair market value.

The Company has not experienced any losses in these accounts and does not believe it is exposed to any significant credit risk on cash and cash equivalents.

Cash, cash equivalents and restricted cash are broken down as follows:

	December 31, 2024	December 31, 2023
Cash	$ 5,640,783	$ 5,027,658
Money Market	4,745,080	6,767,291
Total cash and cash equivalents	$ 10,385,863	$ 11,794,949
Restricted cash	$ 31,695	$ 34,021

Restricted cash refers to an amount required under the Company's office lease agreement in Lugano and is deposited into a restricted bank account as a guarantee.

5. Marketable Securities

As of December 31, 2024 and December 31, 2023, the Company reported nil and $5.0 million of marketable securities, respectively, within current assets, related to United States Treasury Securities ("USTS"). The USTS in the portfolio reached their final maturity in April 2024.

The Company classifies the USTS, which are accounted for as available-for-sale, within the Level 1 fair value hierarchy category as the fair value is based on quoted market prices in active markets with a high level of daily trading volume. Unrealized losses on available-for-sale debt securities as of December 31, 2023 were primarily due to changes in interest rates. The following tables summarize the Company's investment in available-for-sale marketable securities with the detail of the unrealized losses and the estimated fair value as of December 31, 2023:

	December 31, 2023				
	Amortized Cost	Allowance for Credit Losses	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Marketable securities available for sale					
Debt Securities - U.S. government treasury securities	$ 5,004,679	$ —	$ —	$ (4,975)	$ 4,999,704
Totals	$ 5,004,679	$ —	$ —	$ (4,975)	$ 4,999,704

6. Tax Credits, Prepaid Expenses and Other Current Assets

Tax credits, prepaid expenses and other current assets consisted of the following:

	December 31, 2024	December 31, 2023
Tax credits	$ 271,079	$ 242,577
Prepaid and deferred expenses	$ 369,893	$ 608,638
Prepaid directors and officers (D&O) insurance costs	80,959	133,000
Research and development grant receivable	494,684	—
Total prepaid expenses and other current assets	$ 945,536	$ 741,638

Tax credits consisted of a value-added tax credit, which is an indirect tax receivable from Swiss and Spanish tax authorities on purchases of goods and services executed in those countries.

Prepaid expenses refers to pre-payments made to the Company's vendors for future services. Deferred expenses mainly refer to research agreements entered into with third parties for research projects that will be recognized as expenses throughout the research period.

Prepaid D&O insurance costs relate to an annual insurance premium which will be recognized in the statement of operations on a monthly basis throughout the one year insurance period.

The research and development grant receivable relates to a tax credit for eligible research and development costs incurred under the R&DTI program. Refer to Note 3 for further discussion.

7. Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31, 2024	December 31, 2023
Computer	$ 101,087	$ 83,894
Furniture and fixtures	58,573	62,825
Leasehold improvements	31,616	33,992
Laboratory instruments	35,700	38,048
Total property and equipment	226,976	218,759
Less: accumulated depreciation	(123,357)	(92,797)
Property and equipment, net	$ 103,619	$ 125,962

No disposals, nor impairments occurred during the years ended December 31, 2024 and 2023. Depreciation has been calculated by taking into consideration the use, purpose and financial-technical duration of the assets based on their estimated economic lives. Depreciation expense for the years ended December 31, 2024 and December 31, 2023 was $38 thousand and $35 thousand, respectively.

8. Operating Leases

The Company leases offices in Bethesda (United States), Lugano (Switzerland), and Barcelona (Spain). The current lease portfolio consists of leases with remaining terms ranging from less than one to two years. Renewal options are included in the calculation of ROU assets and lease liabilities when the Company is reasonably certain that the renewal options will be exercised. The Company's lease agreements do not contain residual value guarantees or material restrictive covenants.

The breakdown of the significant components of ROU assets, lease liabilities and operating lease expense is reported in the table below, together with the discount rate used in order to calculate the net present value of the lease liabilities as of those periods.

	December 31, 2024	December 31, 2023
Operating lease		
Operating lease right-of-use assets	$ 219,715	$ 459,215
Operating lease liability - current	$ 160,913	$ 229,693
Operating lease liability - noncurrent	$ 53,598	$ 229,855
Weighted average remaining lease term - years	1.38	2.25
Weighted average discount rate	1.53	1.51

The operating lease expenses are reported as follows:

	December 31, 2024	December 31, 2023
Research and development	$ 140,577	$ 141,591
General and administrative	88,499	104,574
Total operating lease costs	$ 229,076	$ 246,165

The Company incurred $17,636 in costs related to the short term lease in Bethesda in the year ended December 31, 2024. The variable lease costs incurred in the years December 31, 2024 and December 31, 2023 was $24,469 and $39,948, respectively.

The Company made fixed cash payments related to operating leases of $0.2 million in both the years ended December 31, 2024 and December 31, 2023.

The future minimum lease payments for the Company's operating leases as of December 31, 2024, are as follows:

Fiscal Year	Operating Leases
2025	$ 162,991
2026	53,973
Total future minimum lease payments	216,964
Less amount representing interest or imputed interest	2,453
Present value of lease liabilities	$ 214,511

9. Accounts Payable

Accounts payable refer to amounts due to third parties on outstanding invoices received for services already provided. As of December 31, 2024 and December 31, 2023, accounts payable amounted to $0.9 million and $1.3 million, respectively. All accounts payable are due in less than 12 months.

10. Other Current Liabilities and Deferred Grant Income

Other current liabilities and deferred grant income consist of the following:

	December 31, 2024	December 31, 2023
Payable for social security and withholding taxes	$ 229,319	$ 368,345
Accrued payroll	1,377,428	726,474
Accrued research and development	344,138	588,433
Accrued professional fees	461,966	399,034
Accrued other	10,417	29,115
Tax provision	18,493	48,965
Total other current liabilities	2,441,761	2,160,366
Deferred grant income	299,652	1,216,924
Total other current liabilities and deferred income	$ 2,741,413	$ 3,377,290

Accrued payroll refers to accruals for year-end bonuses, accrued vacations, overtime and other payroll-related accruals.

Accrued other refers to invoices to be received from vendors for services performed and not yet billed.

Tax provision refers to a tax payable due to the Spanish tax authorities related to taxable income generated in Spain.

Deferred grant income refers to the upfront payment that the Company has received after the successful application regarding research and development grants with Innosuisse.

11. Pension and Other Benefit Programs

Net pension obligations related to the Company's defined pension plan refers only to Swiss employees and as of December 31, 2024 and December 31, 2023 can be summarized as follows:

	December 31, 2024	December 31, 2023
End of year funded status:		
Fair value of plan assets	$ 898,853	$ 822,763
Projected benefit obligation	(1,342,476)	(1,130,217)
Funded status	$ (443,623)	$ (307,454)
Accumulated benefit obligation	$ 1,270,787	$ 1,076,742
Reconciliation of funded status:		
Funded status beginning of year	$ (307,454)	$ (157,580)
Expense	(150,890)	(149,309)
Employer contributions	122,915	143,599
Translation differences	24,330	(16,563)
Change in accumulated other comprehensive loss	(132,524)	(127,601)
Funded status at end of year	$ (443,623)	$ (307,454)
Component of net periodic pension costs:		
Service cost	$ 142,734	$ 144,565
Interest cost	14,836	19,264
Expected return on plan assets	(11,133)	(11,786)
Amortization of losses	8,252	—
Amortization of prior service credit	(3,799)	(2,734)
Total	$ 150,890	$ 149,309

Service cost is reported in general and administrative expenses. Certain other components of net periodic pension costs are reported in interest income, net in the Consolidated Statements of Operations.

	December 31, 2024	December 31, 2023
Reconciliation of projected benefit obligation:		
Projected benefit obligation at January 1	$ 1,130,217	$ 832,707
Service cost	142,734	144,565
Employee contributions	81,930	96,099
Interest cost	14,836	19,264
Benefit payments	(40,044)	(119,897)
Loss on financial assumptions	106,396	132,781
Loss on demographic assumptions	—	2,730
Gain on experience	(12,990)	(61,208)
Translation differences	(87,608)	91,689
Plan amendment	7,005	(8,513)
Total	$ 1,342,476	$ 1,130,217

	December 31, 2024	December 31, 2023
Reconciliation of fair value of plan assets:		
Fair value at January 1	$ 822,763	$ 675,127
Expected return on plan assets	11,133	11,786
Loss on plan assets	(36,566)	(59,077)
Employer contributions	122,915	143,599
Employee contributions	81,930	96,099
Benefit payments	(40,044)	(119,897)
Translation differences	(63,278)	75,126
Fair value at December 31	$ 898,853	$ 822,763

	December 31, 2024	December 31, 2023
Change in net loss:		
Loss at beginning of year	$ 182,921	$ 49,541
Loss on pension benefit obligation during the year	93,406	74,303
Loss on assets during the year	36,566	59,077
Amortization of loss	(8,252)	—
Loss at end of year	$ 304,641	$ 182,921

	December 31, 2024	December 31, 2023
Change in accumulated other comprehensive loss:		
Accumulated other comprehensive income at beginning of year	$ 156,271	$ 28,670
Net loss amortized	(8,252)	—
Loss on pension benefit obligation during the year	93,406	74,303
Loss on assets during the year	36,566	59,077
Prior service cost/(credit) occurring over the year	7,005	(8,513)
Net prior service credit amortized	3,799	2,734
Total accumulated other comprehensive income at end of year	$ 288,795	$ 156,271

The assumptions used in the determination of the benefit obligation and the net periodic costs for the pension plans were as follows:

	December 31, 2024	December 31, 2023
Financial assumptions (%pa):		
Discount rate	0.90%	1.40%
Interest credit rate / expected return on assets	1.25%	1.25%
Salary increases	2.50%	2.50%
Pension increases	0.00%	0.00%
Inflation	1.50%	1.50%
Demographic assumptions:		
Lump-sum option	25%	25%
Retirement age	65/65	65/64
Proportion married	BVG 2020	BVG 2020
Allowance for child pensions	5% loading on risk benefits	5% loading on risk benefits
Mortality base table	BVG 2020	BVG 2020
Longevity improvement	CMI 2018 (1.25%)	CMI 2018 (1.25%)
Turnover	BVG 2020	BVG 2020
Disability	80% BVG 2020	80% BVG 2020

	December 31, 2024	December 31, 2023
Expected benefit payments:		
Year 1	$ 43,568	$ 43,703
Year 2	48,374	50,060
Year 3	52,452	55,679
Year 4	56,008	60,285
Year 5	60,105	64,215
Next 5 years	498,129	482,594
Other disclosure items:		
Next year's expected employer contribution	$ 123,399	$ 156,395

The actuarial losses in 2024 were primarily due to a decrease in discount rate applied against future expected benefit payments and resulted in an increase of the benefit obligation. The increase of the plan assets recorded during the year was mainly related to employer's and employees' contributions to the plan.

The Company's investment strategy for its pension plans is to optimize the long-term investment return on plan assets in relation to the liability structure to maintain an acceptable level of risk while minimizing the cost of providing pension benefits and maintaining adequate funding levels in accordance with applicable rules in each jurisdiction. The Company does not manage any assets internally. The insurance contract plan asset relates to mandatory and discretionary contributions made in accordance with Swiss law to a leading pension provider. The capital is insured and provides for a minimum rate of return. The fair value is equal to the employees' accrued savings and is calculated using total employer and employee contributions plus any accumulated interest credited (which is substantially equivalent to the related cash surrender value). The plan asset has been classified within Level 3 of the fair value hierarchy and the approach is consistent with prior years.

The Company maintains a 401(k) savings plan, which is available to all U.S. employees. Participants may make voluntary contributions. The Company makes matching contributions according to the 401(k) savings plan's matching formula. All matching contributions and participant contribution vest immediately. The expenses related to the Company's 401(k) savings plan consist of matching contributions. Expenses related to the Company's 401(k) savings plan totaled $36,528 and $19,938 for the years ended December 31, 2024 and December 31, 2023.

The Company's United Kingdom ("UK") employees are eligible to participate in its UK defined contribution pension scheme upon commencement of employment. The employees and the Company will make such contributions in line with the rules of the pension scheme in force. The expenses related to the Company's pension scheme consist of matching contributions and totaled $14,563 and $16,753 for the years ended December 31, 2024 and December 31, 2023, respectively.

12. Loans

In August 2020, the Company obtained a CHF 638,000 ($700,221 at the historical exchange rate) nine-year loan, due in quarterly installments with payments commencing on December 31, 2021, and ending on September 30, 2029. The loan was part of the infrastructure put in place by the Federal Council and Swiss Parliament in view of the economic consequences of the COVID-19 pandemic, bears no interest, and had no issuance costs. The Company accounts for this loan at face value, which is deemed to approximate the related fair value.

The future payments under the loan are reported in the table below:

	Total	2025	2026	2027	2028	2029
Loan	$ 438,504	$ 110,177	$ 88,142	$ 88,142	$ 88,142	$ 63,901

13. Fair Value Measurement

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. The Company's assessment of the significance of a particular input to

the fair value measurement in its entirety requires management to make judgments and consider factors specific to the asset or liability.

The carrying amounts of the Company's cash and cash equivalents, including money market funds, restricted cash and financial liabilities are considered to be representative of their respective fair values because of the short-term nature and the contractual terms of those instruments. The fair values of money market funds are based upon the quoted prices in active markets provided by the holding financial institution, which are considered Level 1 inputs in the fair value hierarchy according to ASC 820, "Fair Value Measurement." There have been no changes to the valuation methods utilized by the Company, nor were there transfers between levels of the fair value hierarchy.

	Fair value measurement at reporting date using		
	Quoted prices in active market for identical assets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
December 31, 2024:			
Assets			
Defined benefit pension plan:			
Pension plan asset	$ —	$ —	$ 898,853
Total defined benefit pension plan	—	—	898,853
Cash equivalents:			
Money market funds	4,745,080	—	—
Total cash equivalents	4,745,080	—	—
Total financial assets	$ 4,745,080	$ —	$ 898,853
December 31, 2023:			
Assets			
Marketable securities available for sale:			
Debt securities - U.S. government treasury securities, current	$ 4,999,704	$ —	$ —
Total marketable securities available for sale	4,999,704	—	—
Defined benefit pension plan:			
Pension plan asset	—	—	822,763
Total defined benefit pension plan	—	—	822,763
Cash equivalents:			
Money market funds	6,767,291	—	—
Total cash equivalents	6,767,291	—	—
Total financial assets	$ 11,766,995	$ —	$ 822,763

The carrying amounts of prepaid expenses and other current assets, accounts payable, and accrued expenses approximate fair value due to their short-term maturities. Please refer to Note 11 "Pension Obligations" for additional details on the valuation of pension plan assets.

14. Common Stock, Preferred Stock and Warrants

As of December 31, 2024 and December 31, 2023, the authorized capital stock of the Company included 50,000,000 shares of common stock, $0.0001 par value and 10,000,000 shares of preferred stock, $0.0001 par value. As of December 31, 2024 and December 31, 2023, there were 27,132,588 and 16,206,680 shares of common stock, respectively, $0.0001 par value, issued and outstanding.

At the market offering

In May 2022, the Company entered into a Controlled Equity Offering Sales Agreement (the "Cantor Sales Agreement") with Cantor Fitzgerald & Co. ("Cantor"), pursuant to which the Company was able to sell from time to time, through the agent, Cantor, shares of common stock, having an aggregate offering price of up to $16.0 million (the "ATM Program"). For the year ended December 31, 2023, the Company sold an aggregate of 862,535 shares of common stock under the ATM Program at an average price of $4.60 per share for aggregate gross proceeds of $3.9 million, which included $0.4 million of sales commissions and other offering expenses. The Cantor Sales Agreement was terminated in conjunction with the public offering and concurrent private placement of shares of the Company's common stock in November 2023 as described below.

In September 2024, the Company entered into an Equity Distribution Agreement (the "Distribution Agreement") with Oppenheimer & Co. Inc., serving as agent ("Oppenheimer") with respect to an at-the-market ("ATM") offering program (the "2024 ATM Program"). Under the 2024 ATM Program the Company may offer and sell, from time to time at its sole discretion, shares of common stock having an aggregate offering price of up to $50.0 million. The Company will pay Oppenheimer a commission equal to 3.0% of the gross sales proceeds of any shares sold through Oppenheimer under the Distribution Agreement. During the year ended December 31, 2024, the Company sold an aggregate of 1,597,128 shares of commons stock at an average selling price of $1.97 per share under the 2024 ATM Program, raising gross proceeds of $3.1 million, which included $0.2 million of sales commissions and other offering expenses.

Public and private offering November 2023

In November 2023, the Company completed the public offering of 2,545,000 shares of its common stock and warrants to purchase 1,272,500 shares of its common stock (the "Public Warrants"). The warrants have been offered and sold at the rate of one warrant for every two shares of common stock purchased. The public offering price for each set of two shares of common stock and accompanying warrant to purchase one share of common stock was $4.01, yielding an effective price of $2.00 per share and $0.01 per warrant.

In connection with the public offering that occurred in November 2023, the Company also issued 178,150 warrants to purchase an equal amount of its common stock at an exercise price of $2.75 per share to the underwriter as consideration for the services provided (the "2023 Underwriter Warrants"). The 2023 Underwriter Warrants provide for cashless exercise.

In a private placement that was completed concurrently with the public offering from November 2023 described above, the Company also issued to accredited investors 744,026 shares of its common stock, 1,756,062 pre-funded warrants, to purchase an equal amount of its common stock at the nominal exercise price of $0.0001 and private warrants to purchase 2,500,088 shares of its common stock (the "Private Warrants"). The Private Warrants were sold at the rate of one warrant for every share of common stock (or pre-funded warrant in lieu thereof) purchased in the private placement. The private placement price per share (or pre-funded warrant in lieu thereof) and accompanying Private Warrants to purchase one share of common stock was $2.00 per set of securities sold privately.

In connection with the private placement from November 2023, the Company also issued 175,006 warrants to purchase an equal amount of its common stock at an exercise price of $2.75 to the placement agent as consideration for the services provided. These warrants provide for cashless exercise.

The public offering and the concurrent private placement that were finalized in the fourth quarter of the year ended December 31, 2023, resulted in combined gross proceeds of $10.1 million, which included $1.2 million of underwriting commissions, placement agent's fees and other expenses connected with the financing round.

Public offering June 2024:

In June 2024, the Company completed the public offering of 7,116,547 shares of its common stock and 1,031,602 pre-funded warrants (the "Pre-Funded Warrants") to purchase an equal amount of its common stock at the nominal exercise price of $0.0001. The public offering price was $1.35 per share while the purchase price of each pre-funded warrant was equal to the public offering price at which a share of common stock was sold less $0.0001.

As part of the public offering, the Company granted the underwriter an over-allotment option to purchase up to an additional 1,222,222 shares of its common stock, at the public offering price of $1.35, less underwriting discounts and commissions. In July 2024, the underwriter partially exercised the over-allotment option and purchased an additional 337,076 shares of the Company's common stock at the offering price mentioned above. The over-allotment option expired as of the end of July 2024.

In connection with the public offering, the Company also issued 593,965 warrants to purchase an equal amount of its common stock at an exercise price of $1.6875 per share to the underwriter as a consideration for the services provided (the "2024 Underwriter Warrants"). The 2024 Underwriter Warrants provide for cashless exercise.

The public offering that was finalized in 2024 resulted in gross proceeds of $11.5 million, which include $1.2 million of underwriting commissions and other expenses connected with the financing round. The fair value of the shares of common stock issued in the offering has been recorded in additional paid-in capital and the totality of the gross proceeds has been allocated to the shares of common stock issued.

The fair market value of the Underwriter Warrants that have been issued in connection with the public offering that occurred in fiscal year 2024 has been calculated using the Black-Scholes option pricing model, while the fair market value of the pre-funded warrants has been determined as the spread between the price paid by investors and the closing price of the Company's stock at grant date. The 2024 Underwriter Warrants were recorded within additional paid-in capital, as they represent compensation associated with the financing round, for $0.3 million. Below is a table that summarizes the assumptions that have been used in the calculation:

	Year Ended December 31, 2024	
	2024 Pre-Funded Warrants	2024 Underwriter Warrants
Market price at grant date	$ 1.32	$ 1.32
Volatility	— %	73.47 %
Expected term (years)	—	2.75
Risk-free interest rate	— %	4.28 %
Expected dividend yield	—	—
Grant date fair value per share	$ —	$ 0.56

Warrants:

Below is a summary of the Company's issued and outstanding warrants as of December 31, 2024:

Expiration Date	Weighted Average Exercise Price	Warrants Outstanding	Warrants Exercisable
May 6, 2025	$ 13.75	200,000	200,000
July 20, 2025	$ 5.07	225,387	225,387
November 24, 2028	$ 2.75	4,084,426	4,084,426
June 17, 2029	$ 1.69	593,965	593,965
Pre-funded warrants	$ —	1,031,602	1,031,602
Outstanding as of December 31, 2024	$ 2.63	6,135,380	6,135,380

The following table summarizes the Company's warrants activity for the year ended December 31, 2024:

	Warrants	Weighted Average Exercise Price
Outstanding as of December 31, 2023	6,307,193	$ 2.42
Issued:		
Pre-funded Warrants	1,031,602	$ —
2024 Underwriter Warrants	593,965	$ 1.69
Exercised:		
Pre-funded Warrants	(1,756,062)	$ —
Public Warrants	(41,318)	$ 2.75
Outstanding as of December 31, 2024	6,135,380	$ 2.63

15. Equity Incentive Plan

On September 24, 2020, the Company's Board of Directors (the "Board") adopted the 2020 Omnibus Incentive Plan (the "2020 Omnibus Plan"). On May 12, 2022, the Board approved the Company's 2022 Equity Incentive Plan (the "2022 Plan"), which was approved at the Company's annual meeting of stockholders on June 16, 2022. The 2022 Plan is the successor to, and continuation of, the 2020 Omnibus Plan. The total number of shares reserved for issuance under the 2022 Plan (including shares remaining available under the 2020 Omnibus Plan) is 1,800,000, which increases automatically by 6% every year on January 1 based on the number of shares of common stock issued and outstanding as of the previous year-end. No incentive stock options may be granted under the 2022 Plan after May 12, 2032 and the Board may suspend or terminate the 2022 Plan at any time. The Board is responsible for administering the 2022 Plan.

In addition to the above, on December 23, 2021, the Board adopted the Inducement Equity Incentive Plan (the "2021 Inducement Equity Incentive Plan"), intended to induce new employees to join the Company for the benefit of individuals who satisfy the standards for inducement grants under Rule 5635(c)(4) of the Nasdaq listing rules. The maximum number of shares reserved for issuance pursuant to awards granted under the 2021 Inducement Equity Incentive Plan is 1,000,000.

Stock options are generally granted with a 10 year term at exercise prices equal to the market price at the date of grant. After one year of service from the date of grant, 25% of the options become exercisable, with the remainder becoming exercisable monthly over the following three-year period. RSUs generally vest 25% after one year of service from the date of grant and the remainder vesting quarterly over the following three-year period.

Stock Option Grants

The following table summarizes the Company's stock option activity for the year ended December 31, 2024:

	Shares	Weighted Average Grant Date Fair Value	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (in years)	Aggregate Intrinsic Value
Options outstanding as of December 31, 2023	2,574,299	$ 3.06	$ 4.52	7.70	$ —
Options granted	1,402,500	2.47	3.98		
Options exercised	(15,983)	2.62	3.61		
Options cancelled/forfeited	(808,371)	3.03	4.34		
Options outstanding as of December 31, 2024	3,152,445	$ 2.80	$ 4.33	7.99	$ —
Vested as of December 31, 2024	1,907,132	2.88	4.46	7.39	$ —
Options exercisable as of December 31, 2024	1,907,132	$ 2.88	$ 4.46	7.39	$ —

The aggregate intrinsic value of stock options is calculated as the difference between the weighted average exercise price of the underlying stock options and the market price of the Company's common stock on December 31, 2024. Based on this calculation the intrinsic value of the outstanding stock options as of December 31, 2024 was nil.

As of December 31, 2024, unrecognized compensation costs associated with the stock option grants was $2.9 million and will be recognized over a period of 3.25 years. The fair value of the options vested was $2.1 million for both the years ended December 31, 2024 and December 31, 2023.

The assumptions that the Company used to determine the grant-date fair value of stock options granted during the periods ended December 31, 2024 and December 31, 2023 were as follows, presented on a weighted-average basis:

	Year Ended December 31	
	2024	2023
Grant date fair value	$ 2.47	$ 3.40
Volatility	66 %	77 %
Expected term (years)	5.64	6.60
Risk-free interest rate	4.23 %	3.49 %
Expected dividend yield	—	—

The assumptions that cause the greatest variation in fair value in the Black-Scholes model are the volatility and expected term of exercise. Increases or decreases in either the volatility or expected term of exercise will cause the Black-Scholes option value to increase or decrease, respectively. Each of these inputs is subjective and generally requires significant judgment to determine.

Restricted Stock Units

The following table summarizes the Company's RSUs activity for the year ended December 31, 2024:

	Numbers of Shares	Weighted Average Grant Date Fair Value per Share
Outstanding as of December 31, 2023	493,799	$ 2.54
Granted	25,000	$ 4.37
Vested	(61,794)	$ 4.25
Cancelled/forfeited	(354,303)	$ 1.91
Outstanding as of December 31, 2024	102,702	$ 4.12
Total unrecognized expense remaining	$ 220,455	
Years unrecognized expense expected to be recognized over	2.3	

Options and RSUs do not have voting rights and the underlying shares are not considered issued and outstanding.

The fair value of the RSUs vested was $487 thousand and $328 thousand for the years ended December 31, 2024 and December 31, 2023, respectively. The total stock-based compensation expense for stock options and RSUs, granted to employees and non-employees, has been reported in the Company's Consolidated Statements of Operations as follows:

	Year Ended December 31	
	2024	2023
Research and development	$ 1,020,994	$ 846,571
General and administrative	1,369,165	2,412,455
Total stock-based compensation	$ 2,390,159	$ 3,259,026

16. Income Taxes

The Company is subject to taxation in the U.S., Switzerland, Spain and Australia. Taxes are recorded on an accrual basis and represent the allowances for taxes paid or to be paid for the year, calculated according to the current enacted rates and applicable laws. The Company has accumulated net tax losses since inception in Switzerland and in the U.S. The Company reports a provision for income taxes due to the Spanish and Australian tax authorities pertaining to our subsidiaries Gain Therapeutics Sucursal en España and Gain Therapeutics Australia PTY LTD.

For financial reporting purposes, loss before income tax provision includes the following components:

	Year Ended December 31,	
	2024	2023
Domestic	$ (22,388,925)	$ (22,477,695)
Foreign	2,514,549	289,450
Total	$ (19,874,376)	$ (22,188,245)

The following is the breakdown of the components of income tax expense provision for the years ended December 31, 2024 and 2023:

	Year Ended December 31,	
	2024	2023
Current:		
Federal	$ —	$ —
State	—	—
Foreign	536,815	79,275
Total	$ 536,815	$ 79,275
Deferred:		
Federal	$ —	$ —
State	—	—
Foreign	—	—
Total	—	—
Total income tax expense	$ 536,815	$ 79,275

The breakdown of domestic and foreign net operating losses ("NOLs") and related deferred tax assets are reported in the following table:

	Year Ended December 31,	
	2024	2023
NOLs (domestic)	$ (37,839,738)	$ (27,850,813)
NOLs (foreign)	(9,612,067)	(10,932,386)
Total NOLs	$ (47,451,805)	$ (38,783,199)
Deferred tax assets related to:		
Net operating loss (domestic)	$ 10,411,842	$ 7,662,929
Net operating loss (foreign)	1,730,172	2,001,169
Stock based compensation (domestic)	932,770	820,078
Stock based compensation (foreign)	120,497	96,173
Section 174 - Capitalized R&D	1,573,082	947,754
Warrant expense	278,198	278,198
Patent expense	95,885	103,386
Other temporary differences	167,889	147,245
Total deferred tax assets	$ 15,310,335	$ 12,056,932
Deferred tax liabilities		
Depreciation and other	$ (2,874)	$ (15,977)
Total deferred tax liabilities	$ (2,874)	$ (15,977)
Valuation allowance	$ 15,307,461	$ 12,040,955
Net deferred tax assets	$ —	$ —

Foreign NOLs refer to the Company's Swiss subsidiary and according to Swiss tax law such NOLs can be carried forward for seven years and will begin to expire commencing from 2025 for the NOLs generated in 2017.

According to the U.S. Tax Cuts and Jobs Act ("TCJA") that was signed into law on December 22, 2017, federal NOLs incurred after December 31, 2017 can be carried forward indefinitely and are limited to 80% of taxable income in any tax period. The NOLs and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOLs and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has not done an analysis to determine whether or not ownership changes have occurred since inception.

Deferred tax assets require an assessment of both positive and negative evidence when determining whether it is more likely than not that they can be recovered. Such assessment is made on a jurisdiction-by-jurisdiction basis. The Company's assessment includes an evaluation of cumulative losses, future sources of taxable income and risks and uncertainties related to our business. As of December 31, 2024 and 2023, the Company has determined that there is not sufficient evidence that the Company will be able to realize the benefits of the domestic and foreign deferred tax assets. Accordingly, due to uncertainty regarding their realization, the Company continues to maintain a full valuation allowance on the Company's domestic and foreign deferred tax assets as of December 31, 2024 and 2023 and until sufficient positive evidence will exist to support the reversal of the valuation allowance.

A reconciliation of income tax expense computed at the statutory federal income tax rate to the Company's effective tax rate as reflected in the consolidated financial statements is as follows:

	Year Ended December 31,	
	2024	2023
Federal income tax at U.S. statutory rate	21.00 %	21.00 %
State income taxes, net of federal benefit	4.76 %	2.41 %
Permanent differences	(11.91)%	(9.48)%
Provision to return	0.00 %	(2.80)%
Foreign taxes rate differential	(0.29)%	0.04 %
Valuation allowance	(16.26)%	(11.53)%
Effective income tax rate	(2.70)%	(0.36)%

For the year ended December 31, 2024, permanent differences are mainly attributable to tax on global intangible low-tax income ("GILTI") which was enacted as part of the TCJA. GILTI, in general, is determined annually based on the Company's aggregate foreign subsidiaries' income in excess of certain qualified business asset investment returns. The Company accounts for taxes on GILTI in the period that it is subject to such taxes.

As of December 31, 2024 and 2023, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's Consolidated Statements of Operations. There are no changes expected to occur in the next 12 months with respect to the status of the Company's uncertain tax positions.

The Company files income tax returns in the U.S., Switzerland, Spain and Australia. Tax returns from fiscal year 2017 and onwards remain subject to examination by the taxing jurisdictions. The NOL and tax carryforwards remain subject to review until utilized. The Company is currently not under examination by any tax authorities.

17. Net Loss per Common Share

Basic net loss per common share is computed by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per common share is computed by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock and potentially dilutive securities outstanding during the period. For purposes of the diluted net loss per share calculation, preferred stock, warrants, stock options, RSUs, and PRSUs are considered to be potentially dilutive securities, but are excluded from the calculation of diluted net loss per share because their effect would be anti-dilutive and, therefore, basic and diluted net loss per share are the same for all periods presented.

The following table sets forth the outstanding weighted-average potentially dilutive securities that have been excluded from the calculation of diluted net loss per share because to do so would have resulted in anti-dilutive impacts:

	Year Ended December 31	
	2024	2023
Options to purchase common stock	3,125,122	2,411,327
RSUs and PRSUs	252,250	497,899
Warrants to purchase common stock	4,836,427	843,613

The weighted-average number of warrants to purchase common stock as per the table above does not include the weighted-average effect of 1,031,602 pre-funded warrants for which the exercise price is less than or equal to $0.0001 per share. The weighted-average effect of the pre-funded warrants has been included in the computation of the net loss per share attributable to common stockholders – basic and diluted in the Consolidated Statements of Operations.

18. Related Parties

Dr. Khalid Islam, the Chairman of the Company's Board, shareholder and founder of the Company, is currently the Chairman of the Board of Directors of Minoryx Therapeutics SL ("Minoryx"), and, therefore, Minoryx is considered a related party of the Company. In December 2017, the Company entered into an exclusive worldwide, royalty-bearing, assignable, transferable license agreement with Minoryx to use and exploit Minoryx's intellectual property and into an exclusive worldwide, royalty-bearing, assignable, transferable sublicense agreement with Universitat de Barcelona and Institucio Catalana Recerca Estudis Avancats in order to be able to develop its business, directly or indirectly, through sub-licensing to third parties or any other way of operation. According to the terms and conditions of the Minoryx License Agreement, the Company shall pay to Minoryx as royalties:

- an amount equal to 8% of (i) net revenues with regard to products that would infringe (a) at least one composition of matter claim or (b) Minoryx molecules and (ii) sublicensing revenues; and

- an amount equal to 3% of net revenues with regard to products that would infringe at least (a) one method of claim or (b) Minoryx know-how (as such term is defined in the agreement).

As of December 31, 2024 and December 31, 2023, there were no receivables and payables, revenues or expenses with Minoryx.

On September 20, 2022 the Company entered into a consulting agreement with Mr. Eric Richman, who previously served as the Company's CEO and is currently a member of the Board of Directors of the Company. As per the consulting agreement Mr Richman agreed to provide consulting services as a special advisor to the Company and its Board of Directors at the request of the Chairman of the Board or another member of the Board of the Company. The consulting agreement terminated on September 20, 2023. In connection with this consulting agreement during the year ended December 31, 2023 the Company incurred expenses of $236 thousand. As of December 31, 2024 and December 31, 2023, there were no receivables or payables connected with the consulting agreement with Mr. Richman.

19. Commitments and Contingencies

Commitments:

As of December 31, 2024, the Company had research commitments for $4.2 million for activities that will be performed within one year.

Contingencies:

The Company records a provision for its contingent obligations when it is probable that a loss will be incurred, and the amount of the loss can be reasonably estimated. On September 18, 2024, Matthias Alder ("Alder"), the Company's former Chief Executive Officer, filed litigation against the Company in the Circuit Court of Maryland for Montgomery County. Mr. Alder's employment was terminated on June 25, 2024. In connection with the litigation, as of December 31, 2024, the Company has made an accrual of $0.53 million, reported within payroll related accruals.

20. Subsequent Events

From January 1, 2025, through March 24, 2025, the Company has sold an aggregate of 1,062,804 shares of common stock through the "2024 ATM Program" at a weighted-average selling price of $2.28 for total gross proceeds of $2.4 million.

On March 20, 2025, 454,893 pre-funded warrants were exercised resulting in the issuance of 454,893 shares of common stock.

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

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (i) recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms and (ii) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure. Our management, with the participation of our Chief Executive Officer and Principal Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2024. Based upon the evaluation, our Chief Executive Officer and Principal Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost benefit relationship of possible controls and procedures.

Management's Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as such term as defined in Exchange Act Rule 13a-15(f) and 15d-15(f). Internal control over financial reporting is a process designed by, or under the supervision of, our principal executive officer and principal financial officer, or persons performing similar functions, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States of America.

As of December 31, 2024, our management assessed the effectiveness of our internal control over financial reporting using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in Internal Control-Integrated Framework (2013). Based on this assessment, our management concluded that, as of December 31, 2024, our internal control over financial reporting was effective.

Attestation Report of Independent Registered Public Accounting Firm

This Annual Report does not include an attestation report of our independent registered public accounting firm regarding internal controls over financial reporting. We are not required to engage our independent audit firm to perform an audit of the effectiveness of our internal controls over financial reporting for as long as we are an "emerging growth company" pursuant to the provisions of the JOBS Act.

Changes in Internal Control Over Financial Reporting

There have been no other changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act) that occurred during the fourth quarter of 2024 that have materially affected, or are reasonably likely to materially affect, the Company's internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

(a) On March 24, 2025, we entered into an Employment Agreement with Gianluca Fuggetta, our Principal Financial Officer (the "Fuggetta Employment Agreement"). Pursuant to the Fuggetta Employment Agreement, Mr. Fuggetta is entitled to an annual base salary of CHF 200,000 and is eligible for an annual incentive cash bonus with a target payout of 30% of his annual base salary. Further, if we terminate Mr. Fuggetta without good cause, we will pay to Mr. Fuggetta severance equal to six months base salary. If we terminate Mr. Fuggetta within 12 months following a change in control without good cause, the gross severance will equal to 12 months of his annual base salary. The foregoing description of the Fuggetta Employment Agreement does not purport to be complete and is qualified in its entirety by reference to the Fuggetta Employment Agreement, a copy of which is filed as Exhibit 10.18 to this Annual Report on Form 10-K and is incorporated by reference herein.

(b) During the fiscal quarter ended December 31, 2024, no director or "officer" (as defined in Rule 16a-1(f) under the Exchange Act) of the Company adopted or terminated any "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement" as each term is defined in Item 408(c) of Regulation S-K.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS, AND CORPORATE GOVERNANCE

Information required by this item is incorporated by reference to the information set forth in the sections titled "Information about Our Board of Directors" and "Information about Our Executive Officers", "Corporate Governance, "Delinquent Section 16(a) Reports", and "Corporate Governance – Committees of the Board of Directors" in our definitive proxy statement to be filed with the SEC on Schedule 14A in connection with our 2025 Annual Meeting of Shareholders, or the Proxy Statement, which is expected to be filed no later than 120 days after December 31, 2024. If the Proxy Statement is not filed with the SEC within 120 days after the end of our most recent fiscal year, we will provide such information by means of an amendment to this Annual Report on Form 10-K.

ITEM 11. EXECUTIVE COMPENSATION

Information required by the items is incorporated by reference to the information set forth in the sections titled "Executive Compensation" and "Director Compensation" in the Proxy Statement.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Information required by this item is incorporated by reference to the information set forth in the sections titled "Securities Authorized for Issuance Under Equity Compensation Plans" and "Security Ownership of Certain Beneficial Owners and Management" in the Proxy Statement.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

Information required by this item is incorporated by reference to the information set forth in the sections titled "Information Regarding the Board of Directors and Corporate Governance – Independence of the Board of Directors" and "Transactions with Related Persons" in the Proxy Statement.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

Information required by this item is incorporated by reference to the information set forth in the section titled "Independent Registered Public Accounting Firm Fees and Services" in the Proxy Statement.

Exhibit Number	Exhibit	Incorporated by Reference			
		Form	File No.	Exhibit	Filing Date
3.1	Amended and Restated Certificate of Incorporation of Gain Therapeutics, Inc.	8-K	001-40237	3.1	3/17/2021
3.2	Amended and Restated Bylaws of Gain Therapeutics, Inc.	8-K	001-40237	3.2	3/17/2021
4.1*	Description of Securities.				
4.2	Investors' Rights Agreement, dated as of July 20, 2020, by and among Gain Therapeutics, Inc. and certain holders of its capital stock.	S-1	333-253303	4.2	2/19/2021
4.3	Form of Warrant.	8-K	001-40237	4.1	11/22/2023
4.4	Form of Private Warrant.	8-K	001-40237	4.2	11/22/2023
4.5	Form of Underwriter's Warrant.	8-K	001-40237	4.3	11/22/2023
4.6	Form of Pre-Funded Warrant.	8-K	001-40237	4.4	11/22/2023
4.7	Warrant Agent Agreement by and between Gain Therapeutics, Inc. and Pacific Stock Transfer Company.	8-K	001-40237	4.5	11/22/2023
4.8	Form of Placement Agent Warrant.	8-K	001-40237	4.6	11/22/2023
10.1+	Form of Indemnification Agreement for Officers and Directors.	S-1/A	333-253303	10.2	3/10/2021
10.2+	2020 Omnibus Incentive Plan.	S-1/A	333-253303	10.1	3/10/2021
10.3+	Gain Therapeutics, Inc. 2021 Inducement Equity Incentive Plan.	8-K	001-40237	10.1	12/28/2021
10.4+	Form of Stock Option Agreement under the 2021 Inducement Plan.	8-K	001-40237	10.2	12/28/2021
10.5	Minoryx Agreement between Minoryx Therapeutics, S.L. and GT Gain Therapeutics SA.	S-1	333-253303	10.3	2/19/2021
10.6	Form of Exchange Agreement.	S-1	333-253303	10.10	2/19/2021
10.7	Form of Placement Agent Warrant.	S-1	333-253303	10.11	2/19/2021
10.8 +	Gain Therapeutics Inc. 2021 Inducement Equity Incentive Plan Restricted Stock Unit Award Agreement.	S-8	333-266142	4.5	7/15/2022
10.9+	Gain Therapeutics Inc. 2022 Equity Incentive Plan.	S-8	333-266142	4.6	7/15/2022
10.10+	Gain Therapeutics Inc. RSU Award Grant Notice and Award Agreement (2022 Equity Incentive Plan).	S-8	333-266142	4.7	7/15/2022
10.11+	Gain Therapeutics Inc. Stock Option Grant Notice and Award Agreement (2022 Equity Incentive Plan).	S-8	333-266142	4.8	7/15/2022
10.12	Form of Indemnification Agreement for Officers and Directors.	10-Q	001-40237	10.3	8/10/2023
10.13	Form of Indemnification Agreement for Officers and Directors.	10-Q	001-40237	10.3	11/14/2023
10.14	Form of Securities Purchase Agreement.	8-K	001-40237	10.1	11/22/2023
10.15+	Separation Agreement, by and between the Company and Matthias Alder, dated June 27, 2024.	10-Q	001-40237	10.2	11/14/2024
10.16	Distribution Agreement, by and between the Company and Oppenheimer & Co. Inc., dated September 6, 2024	8-K	001-40237	1.1	9/6/2024
10.17+	Amended and Restated Employment Agreement by and between the Company and Gene Mack, dated January 6, 2025.	8-K	001-40237	10.1	1/7/2025
10.18+*	Employment Agreement, by and between the Company and Gianluca Fuggetta, dated March 24, 2025				
19.1*	Insider Trading Policy				
21.1*	Subsidiaries of Registrant.				
23.1*	Consent of Independent Registered Public Accounting Firm.				
24.1*	Power of Attorney (included in the signature pages attached to this Annual Report on Form 10-K).				
31.1*	Section 302 Certification of Chief Executive Officer.				
31.2*	Section 302 Certification of Principal Financial Officer.				
32.1**	Section 906 Certification of Chief Executive Officer and Principal Financial Officer.				
97.1+	Clawback Policy	10-K	001-40237	97.1	3/26/2024

Exhibit Number	Exhibit	Incorporated by Reference			
		Form	File No.	Exhibit	Filing Date
101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.				
101.SCH*	Inline XBRL Taxonomy Extension Schema Document.				
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document.				
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document.				
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document.				
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document.				
104*	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).				

+ Management contract or compensatory plan or arrangement.
* Filed herewith
** This certification is deemed not filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended.

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, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

GAIN THERAPEUTICS, INC.
(Registrant)

Date: March 27, 2025

By: /s/ Gene Mack
Gene Mack
Chief Executive Officer
(*Principal Executive Officer*)

Date: March 27, 2025

By: /s/ Gianluca Fuggetta
Gianluca Fuggetta
Senior Vice President Finance
(*Principal Financial Officer and Principal Accounting Officer*)

POWER OF ATTORNEY

We, the undersigned directors and officers of Gain Therapeutics, Inc., hereby severally constitute and appoint Gene Mack and Gianluca Fuggetta, and each of them singly, our true and lawful attorneys, with full power to them, and to each of them singly, to sign for us and in our names in the capacities indicated below, all amendments to this Annual Report, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as each of us might or could do in person, and hereby ratifying and confirming all that said attorneys, and each of them, or their substitute or substitutes, shall do or cause to be done by virtue of this Power of Attorney.

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

Dated: March 27, 2025	/s/ Gene Mack Gene Mack Chief Executive Officer and Director (Principal Executive Officer)
Dated: March 27, 2025	/s/ Gianluca Fuggetta Gianluca Fuggetta Senior Vice President Finance (Principal Financial Officer and Principal Accounting Officer)
Dated: March 27, 2025	/s/ Khalid Islam Khalid Islam Chairman of the Board of Directors
Dated: March 27, 2025	/s/ Dov Goldstein Dov Goldstein Director
Dated: March 27, 2025	/s/ Hans Peter Hasler Hans Peter Hasler Director
Dated: March 27, 2025	/s/ Gwen Melincoff Gwen Melincoff Director
Dated: March 27, 2025	/s/ Claude Nicaise Claude Nicaise Director
Dated: March 27, 2025	/s/ Eric I. Richman Eric I. Richman Director
Dated: March 27, 2025	/s/ Jeffrey Riley Jeffrey Riley Director

EXHIBIT 4.1

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES ACT OF 1934

The following is a description of the common stock, par value $0.0001 per share (the "Common Stock") of Gain Therapeutics, Inc. (the "Company") which is the only security of the Company registered pursuant to Section 12 of the Securities Exchange Act of 1934, as amended (the "Exchange Act").

General

The Company is authorized to issue 50,000,000 shares of Common Stock, par value $0.0001 per share, and 10,000,000 shares of preferred stock, par value $0.0001 per share. Unless our board of directors determines otherwise, we will issue all shares of our capital stock in uncertificated form.

The following description summarizes selected information regarding the Common Stock, as well as relevant provisions of: (i) the Company's Restated Certificate of Incorporation, as amended, as currently in effect, (ii) the Company's Amended and Restated bylaws, as currently in effect and (iii) the Delaware General Corporation Law (the "DGCL"). The following summary description of the Common Stock is qualified in its entirety by, and should be read in conjunction with, the Articles and the bylaws, copies of which have been filed as exhibits to the Company's periodic reports under the Exchange Act, and the applicable provisions of the DGCL.

Common Stock

The following description of certain rights of our common stock does not purport to be complete and is qualified in its entirety by reference to our amended and restated certificate of incorporation and our amended and restated bylaws.

Voting Rights. Holders of shares of our common stock are entitled to one vote for each share held of record on all matters on which stockholders are entitled to vote generally, including the election or removal of directors elected by our stockholders generally. The holders of our common stock do not have cumulative voting rights in the election of directors.

Dividends and Liquidation Rights. Holders of shares of our common stock are entitled to receive dividends when, as and if declared by our board of directors out of funds legally available therefor, subject to any statutory or contractual restrictions on the payment of dividends and to any restrictions on the payment of dividends imposed by the terms of any outstanding preferred stock. Upon our liquidation, dissolution or winding up and after payment in full of all amounts required to be paid to creditors and to the holders of preferred stock having liquidation preferences, if any, the holders of shares of our common stock will be entitled to receive our remaining assets available for distribution on a pro rata basis.

Miscellaneous. All shares of our common stock that will be outstanding are fully paid and non-assessable. The common stock will not be subject to further calls or assessments by us.

Holders of shares of our common stock do not have preemptive, subscription, redemption or conversion rights. There will be no redemption or sinking fund provisions applicable to the common stock. The rights powers, preferences and privileges of our common stock will be subject to those of the holders of any shares of our preferred stock or any other series or class of stock we may authorize and issue in the future.

Listing. Our Common Stock is listed on the Nasdaq Global Market under the symbol "GANX."

Transfer Agent and Registrar. The transfer agent and registrar for our Common Stock is Philadelphia Stock Transfer, Inc.

Preferred Stock

Voting Rights. No shares of preferred stock are issued or outstanding as of December 31, 2021. Our Amended Charter authorizes our board of directors to establish one or more series of preferred stock (including convertible preferred stock). Unless required by law or any stock exchange, the authorized shares of preferred stock will be available for issuance without further action by the holders of our common stock. Our board of directors is able to determine, with respect to any series of preferred stock, the powers (including voting powers), preferences and relative, participating, optional or other special rights, and the qualifications, limitations or restrictions thereof, including, without limitation:

- the designation of the series;
- the number of shares of the series, which our board of directors may, except where otherwise provided in the preferred stock designation, increase (but not above the total number of authorized shares of the class) or decrease (but not below the number of shares then outstanding);
- whether dividends, if any, will be cumulative or non-cumulative and the dividend rate of the series;
- the dates at which dividends, if any, will be payable;
- the redemption or repurchase rights and price or prices, if any, for shares of the series;
- the terms and amounts of any sinking fund provided for the purchase or redemption of shares of the series;
- the amounts payable on shares of the series in the event of any voluntary or involuntary liquidation, dissolution or winding-up of our affairs;
- whether the shares of the series will be convertible into shares of any other class or series, or any other security, of us or any other entity, and, if so, the specification of the other class or series or other security, the conversion price or prices or rate or rates, any rate adjustments, the date or dates as of which the shares will be convertible and all other terms and conditions upon which the conversion may be made;
- restrictions on the issuance of shares of the same series or of any other class or series; and
- the voting rights, if any, of the holders of the series.

We could issue a series of preferred stock that could, depending on the terms of the series, impede or discourage an acquisition attempt or other transaction that some, or a majority, of the holders of our common stock might believe to be in their best interests or in which the holders of

our common stock might receive a premium over the market price of the shares of our common stock. Additionally, the issuance of preferred stock may adversely affect the rights of holders of our common stock by restricting dividends on the common stock, diluting the voting power of the common stock or subordinating the liquidation rights of the common stock. As a result of these or other factors, the issuance of preferred stock could have an adverse impact on the market price of our common stock.

Anti-Takeover Effects of Our Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws and Certain Provisions of Delaware Law

Our Amended Charter, Amended Bylaws and the DGCL contains provisions that are intended to enhance the likelihood of continuity and stability in the composition of our board of directors. These provisions are intended to avoid costly takeover battles, reduce our vulnerability to a hostile or abusive change of control and enhance the ability of our board of directors to maximize stockholder value in connection with any unsolicited offer to acquire us. However, these provisions may have an anti-takeover effect and may delay, deter or prevent a merger or acquisition of our company by means of a tender offer, a proxy contest or other takeover attempt that a stockholder might consider in its best interest, including those attempts that might result in a premium over the prevailing market price for the shares of common stock held by stockholders.

Authorized but Unissued Capital Stock

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the listing standards of the Nasdaq Global Market. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Business Combinations

We are subject to Section 203 of the DGCL, which prohibits persons deemed to be "interested stockholders" from engaging in a "business combination" with a publicly held Delaware corporation for three years following the date these persons become interested stockholders unless the business combination is, or the transaction in which the person became an interested stockholder was, approved in a prescribed manner or another prescribed exception applies. Generally, an "interested stockholder" is a person who, together with affiliates and associates, owns, or within three years prior to the determination of interested stockholder status did own, 15% or more of a corporation's voting stock. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the interested stockholder. The existence of this provision may have an anti-takeover effect with respect to transactions not approved in advance by the board of directors.

No Cumulative Voting

Under Delaware law, the right to vote cumulatively does not exist unless the certificate of incorporation specifically authorizes cumulative voting. Our Amended Charter does not authorize

cumulative voting. Therefore, stockholders holding a majority of the shares of our stock entitled to vote generally in the election of directors will be able to elect all our directors.

Special Stockholder Meetings

Our Amended Charter provides that special meetings of our stockholders may be called at any time only by or at the direction of the board of directors or the chairman of the board of directors. Our Amended Bylaws prohibit the conduct of any business at a special meeting other than as specified in the notice for such meeting. These provisions may have the effect of deferring, delaying, or discouraging hostile takeovers, or changes in control or management of our company.

Director Nominations and Stockholder Proposals

Our amended and restated bylaws establish advance notice procedures with respect to stockholder proposals and the nomination of candidates for election as directors, other than nominations made by or at the direction of the board of directors or a committee of the board of directors. In order for any matter to be "properly brought" before a meeting, a stockholder will have to comply with advance notice requirements and provide us with certain information. Generally, to be timely, a stockholder's notice must be received at our principal executive offices not less than 90 days nor more than 120 days prior to the first anniversary date of the immediately preceding annual meeting of stockholders. Our Amended Bylaws also specify requirements as to the form and content of a stockholder's notice. Our Amended Bylaws allow the chairman of the meeting at a meeting of the stockholders to adopt rules and regulations for the conduct of meetings that may have the effect of precluding the conduct of certain business at a meeting if the rules and regulations are not followed. These provisions may also defer, delay, or discourage a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to influence or obtain control of the company.

Stockholder Action by Written Consent

Pursuant to Section 228 of the DGCL, any action required to be taken at any annual or special meeting of the stockholders may be taken without a meeting, without prior notice, and without a vote if a consent or consents in writing, setting forth the action so taken, is or are signed by the holders of outstanding stock having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares of our stock entitled to vote thereon were present and voted, unless our amended and restated certificate of incorporation will provide otherwise. Our Amended Charter will preclude stockholder action by written consent at any time.

Amendment of Amended and Restated Certificate of Incorporation or Bylaws

The DGCL provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our bylaws may be amended or repealed by a majority vote of our board of directors or by the affirmative vote of the holders of at least 66 2/3% of the votes which all our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 66 2/3% of the votes which all our stockholders would be entitled to cast in any

election of directors will be required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate described above.

The foregoing provisions of our Amended Charter and Amended Bylaws could discourage potential acquisition proposals and could delay or prevent a change in control. These provisions are intended to enhance the likelihood of continuity and stability in the composition of our board of directors and in the policies formulated by our board of directors and to discourage certain types of transactions that may involve an actual or threatened change of control. These provisions are designed to reduce our vulnerability to an unsolicited acquisition proposal. The provisions also are intended to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and, as a consequence, they also may inhibit fluctuations in the market price of our shares of common stock that could result from actual or rumored takeover attempts. Such provisions also may have the effect of preventing changes in our management or delaying or preventing a transaction that might benefit you or other minority stockholders.

Dissenters' Rights of Appraisal and Payment

Under the DGCL, with certain exceptions, our stockholders will have appraisal rights in connection with a merger or consolidation of us. Pursuant to the DGCL, stockholders who properly request and perfect appraisal rights in connection with such merger or consolidation will have the right to receive payment of the fair value of their shares as determined by the Delaware Court of Chancery.

Stockholders' Derivative Actions

Under the DGCL, any of our stockholders may bring an action in our name to procure a judgment in our favor, also known as a derivative action, provided that the stockholder bringing the action is a holder of our shares at the time of the transaction to which the action relates or such stockholder's stock thereafter devolved by operation of law.

Exclusive Forum

Our Amended Charter will provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will, to the fullest extent permitted by applicable law, be the sole and exclusive forum for: (1) any derivative action or proceeding brought on our behalf; (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or other employees or stockholders to us or our stockholders; (3) any action asserting a claim against us, any director or our officers and employees arising pursuant to any provision of the DGCL, our Amended Charter or our amended and restated bylaws, or as to which the DGCL confers exclusive jurisdiction on the Court of Chancery; or (4) any action asserting a claim against us, any director or our officers or employees that is governed by the internal affairs doctrine; provided that the exclusive forum provisions will not apply to suits brought to enforce any liability or duty created by the Exchange Act, or to any claim for which the federal courts have exclusive jurisdiction. Our Amended Charter further provides that, unless we consent in writing to the selections of an alternative forum, the federal district courts are the sole and exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act, subject to a final adjudication in the State of Delaware of the enforceability of

such exclusive forum provision. We note that investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder. Although we believe the provision benefits us by providing increased consistency in the application of Delaware law in the types of lawsuits to which it applies, the provision may have the effect of discouraging lawsuits against our directors and officers.

Officers and Directors

The DGCL authorizes corporations to limit or eliminate the personal liability of directors to corporations and their stockholders for monetary damages for breaches of directors' fiduciary duties, subject to certain exceptions. Our Amended Charter includes a provision that eliminates the personal liability of directors for monetary damages to the corporation or its stockholders for any breach of fiduciary duty as a director, except to the extent such exemption from liability or limitation thereof is not permitted under the DGCL. The effect of these provisions is to eliminate the rights of us and our stockholders, through stockholders' derivative suits on our behalf, to recover monetary damages from a director for breach of fiduciary duty as a director, including breaches resulting from grossly negligent behavior. However, exculpation does not apply to any breaches of the director's duty of loyalty, any acts or omissions not in good faith or that involve intentional misconduct or knowing violation of law, any authorization of dividends or stock redemptions or repurchases paid or made in violation of the DGCL, or for any transaction from which the director derived an improper personal benefit.

Our Amended Bylaws generally provide that we must indemnify and advance expenses to our directors and officers to the fullest extent authorized by the DGCL. We also are expressly authorized to carry directors' and officers' liability insurance providing indemnification for our directors, officers and certain employees for some liabilities. We believe that these indemnification and advancement provisions and insurance are useful to attract and retain qualified directors and executive officers.

The limitation of liability, indemnification and advancement provisions in our Amended Charter and Amended Bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duty. These provisions also may have the effect of reducing the likelihood of derivative litigation against directors and officers, even though such an action, if successful, might otherwise benefit us and our stockholders. In addition, your investment may be adversely affected to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

There is currently no pending material litigation or proceeding involving any of our directors, officers or employees for which indemnification is sought.

Indemnification Agreements

We entered into an indemnification agreement with each of our directors and executive officers. Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors or executive officers, we have been informed that in the opinion of the SEC such indemnification is against public policy and is therefore unenforceable.

EMPLOYMENT CONTRACT
between

GT Gain Therapeutics SA

Via Francesco Soave 6, CH-6900 Lugano, Switzerland (hereinafter **"Employer")**

and

Gianluca Fuqqetta

[ ], Italian citizen (hereinafter **"Employee")**

Each of them hereinafter a "**Party**" and together the "**Parties".**

The Parties enter into the following employment contract ("**Contract**"):

1. **Start Date:** This Contract shall enter into force on January 1, 2025. July 1, 2022, shall remain the start date to calculate the Employee's seniority with the Employer.

2. **Duration:** Undetermined

3. **Level of Employment:** 100%

4. **Place of Work:** At the Employer's seat, currently in Lugano, with the ability to work from home, depending on business needs and provided the Employee works more than 75% of the working time outside his home jurisdiction. The Employee will be liable for and himself bear and hold the Employer harmless from any damages that a violation of this obligation would trigger. Professional travel may be required.

5. **Function and Mansion:** Senior Vice President Finance. Functions as per attached job description. The Employee acknowledges and accepts that he is at the same time appointed as Principal Financial Officer and Principal Accounting Officer for Gain Therapeutics, Inc. and that no additional remuneration will be paid for this position.

6. **Working Time**: The work week for 100% employment is 40 hours, divided over five working days. In view of the type of function, however, the Employee shall organize himself in the specific case freely so as to achieve the set objectives and to enable the Employer to achieve the business objectives. Any overtime compensation is excluded.

7. **Vacation:** twenty (25) working days for each full calendar year. Vacations are to be agreed upon in advance with the Employer. In the case of employment for a period of less than one year, vacation will accrue pro rata temporis.

8. **Notice Period:** 3 (three) months, valid for both Parties.

9. **Severance:** Provided that the Employer terminates the Employee without the Employee having given the Employer good cause (in accordance with art. 340c para. 2 Swiss Code of Obligations) to do so, the Employer shall pay to the Employee a gross severance equal to six monthly base salaries. If the Employer, within 12 months following a change in control, gives notice of termination to the Employee without the Employee having given the Employer good cause (in accordance with art. 340c para. 2 Swiss Code of Obligations) to do so, the gross severance shall be increased by another six monthly base salaries to a total of 12 monthly base salaries. For the purpose of this provision, a change in control shall exist if:

   (a) any person or any group of persons directly or indirectly purchases or otherwise becomes the beneficial owner or has the right to acquire such beneficial ownership of voting securities representing 50 % or more of the combined voting power of all outstanding voting securities of the Employer; or

   (b) the stockholders of the Employer approve an agreement to merge or consolidate the Employer with or into another corporation (and such other corporation also approves such agreement) as a result of which less than 50% of the outstanding voting securities of the surviving or resulting entity are or will be owned by the former

stockholders of the Employer; or

(c) the stockholders of Employer approve the sale of all or substantially all of Employer's business and/or assets to a person or entity which is not a wholly owned subsidiary of the Employer.

10. **Salary:** The base salary for an employment at 100% is set at CHF 200,000 (two hundred thousand Swiss Francs) gross per year, payable in 12 equal monthly instalments.

11. **Expense Reimbursement:** The Employee shall be compensated for any expenses that are necessary for work activities.

12. **Bonus:** The Employee shall be entitled to a discretionary annual bonus of up to 30% of his annual base salary.

13. **Social Security:** The contributions provided by statutory law and applicable regulations for AVS-AHPG / AD, occupational pension (LPP) and LAINF are paid by the Employer and the Employee in accordance with the law and applicable regulations.

14. **Confidentiality:** During and after the end of the employment relationship, the Employee shall not use or disclose confidential facts, in particular manufacturing or business secrets, of which he has become aware in the service of the Employer.

15. **Non-Compete Obligation:** The Parties note that the Employee as a result of his activities receives information about manufacturing and development secrets and that the use of this knowledge in a competing company could significantly harm the Employer. In the event of violation of the non-competition clause, a conventional penalty equal to half of the last annual salary will be payable, subject, however, to compensation for any greater damage. Pursuant to Art. 340b para. 3 CO, it is explicitly agreed that the payment of the conventional penalty and compensation for any greater damages does not absolve the Employee from the immediate termination of the injurious state.

16. **Return of documents:** At the end of the employment and prior to leaving the work place, all entrusted material and equipment (laptops, prospectuses, customer lists, tools, keys, etc.) have to be returned. Any retention right is excluded.

17. **Amendments:** Any amendments of this Contract have to be in writing and duly signed by both Parties to be valid. An electronic signature shall be sufficient.

18. **Applicable Law:** This Contract shall be subject to Swiss law.

19. **Jurisdiction:** All disputes arising out of or in connection with this Agreement, including any disputes regarding its validity or its termination as well as regarding the validity of this jurisdiction clause, shall be decided by the courts at the domicile or seat of the respondent or at the place where the Employee usually performs the Employee's work.

March 24, 2025

The Employer

GT Gain Therapeutics SA

/s/ Khalid Islam

Khalid Islam
Chairman of the Board

March 24, 2025

The Employee

/s/ Gianluca Fuggetta

Gianluca Fuggetta

Job Description: Employee: (i) shall serve as the Employer's Senior Vice President, Finance, Principal Accounting Officer and Principal Financial Officer, with responsibilities, duties, and authority usual and customary for such positions, subject to direction by the Employer's Chief Executive Officer ("CEO"); (ii) shall report directly to the CEO; and (iii) shall be subject to the supervision of, and shall have such authority as is delegated to him by, the Employer's board of directors and/ or CEO, which authority shall be sufficient to perform his duties hereunder. Employee shall devote his best efforts in the performance of the foregoing.

Exhibit 19.1

Adopted by the Board of Directors on March 20, 2025.

GAIN THERAPEUTICS, INC.
INSIDER TRADING POLICY

INTRODUCTION

As a public company, one of our important ethical duties is to protect and properly use nonpublic information acquired during our service with Gain Therapeutics, Inc. (together with its subsidiaries, "***Gain***" or the "***Company***"). This Insider Trading Policy (the "***Policy***") provides detailed information on these obligations. The rules relating to insider trading are complex, and a violation of insider trading laws can carry severe consequences, including but not limited to termination of your employment and criminal prosecution resulting in imprisonment. In furtherance of the Policy's goals, the Company will not transact in its own securities except in compliance with securities laws.

This introduction provides high level guidance on certain required and prohibited activities. However, carefully review this entire Policy before completing any transaction involving the Company's securities or the securities of any other company.

The following activities, among others, are prohibited under this Policy and may also be illegal:

- Trading "on the basis" of material nonpublic information (see Part II below for more information about what may constitute material nonpublic information)
- "Tipping" or providing material nonpublic information to another person who then trades based on such information. This includes providing trading advice or opinions on transactions
- Making "short sales" of Gain securities (betting that the price of securities will decline)
- Engaging in transactions involving publicly-traded options, such as puts and calls, and other derivative securities with respect to Gain's securities
- Placing "open orders" (such as limit orders or stop orders) with brokers which may remain outstanding for an extended period of time and, as a result, could be executed at a time when you are aware of material nonpublic information or otherwise not permitted to trade
- Trading during Trading Blackout Periods, or during any special blackout periods applicable to you
- Using Gain securities as collateral for loans if you are subject to any Trading Blackout Periods or subject to Section 16 of the Securities Exchange Act of 1934, as amended ("***Section 16***")
- Holding Gain securities in margin accounts if you are subject to any Trading Blackout Periods or subject to Section 16
- Otherwise trading in Gain securities without pre-clearance

Any of the above actions will be deemed violations of this Policy and may result in severe consequences, including but not limited to termination of your employment and criminal prosecution resulting in imprisonment. Prosecutors pursue insider trading violations vigorously, even if the size of the transaction is small.

Even if a transaction is not listed above, you are ultimately responsible for ensuring that it otherwise complies with this Policy and applicable laws and regulations. You should use your best judgment at all times and consult with your personal legal and financial advisors, as needed. Share this policy with your broker or other financial advisor before engaging in any transactions involving Gain's securities or the

securities of any other company. Please seek assistance from the Compliance Officer (as defined below) if you have any questions at all.

PART I

PREVENTING INSIDER TRADING AND YOUR OBLIGATIONS

1. Covered Persons

This Policy applies to all directors, officers, employees, consultants, contractors and other agents of the Company. References in this Policy to such persons, or to "***you***," also include your immediate family members, members of your household and your economic dependents, along with any other individuals or entities whose transactions in securities you influence, direct or control (which may be, for example, a venture or other investment fund). You are responsible for making sure that these other individuals and entities comply with this Policy.

You are expected to comply with this Policy until such time as you are no longer affiliated with the Company *and* you no longer possess any material nonpublic information subject to this Policy, as described in Part II (Material Nonpublic Information). In addition, if you are subject to a trading blackout under this Policy at the time you cease to be affiliated with the Company, you are expected to abide by the applicable trading restrictions until at least the end of the relevant Trading Blackout Period.

2. Covered Activities

Except as discussed in Part III (Limited Exceptions), this Policy applies to all transactions involving the Company's securities or other companies' securities on the basis of material nonpublic information obtained in connection with your service with the Company. This Policy therefore applies to:

- transactions involving the securities of the Company, whether direct or indirect (including transactions made on your behalf by money managers), including purchases, sales and other transfers of common stock, options, warrants, preferred stock and debt securities (such as debentures, bonds and notes);
- transactions involving the securities of other companies on the basis of material nonpublic information obtained in the course of your service with the Company;
- arrangements that affect economic exposure to changes in the prices of these securities, such as transactions in derivative securities (e.g., exchange-traded put or call options), hedging transactions, short sales and certain decisions with respect to participation in benefit plans;
- any disposition in the form of a gift of any securities of the Company;
- any distribution to holders of interests in an entity if the entity is subject to this Policy;
- any offers with respect to the transactions discussed above; and
- other unauthorized use or disclosure of nonpublic information.

There may be instances where you suffer financial harm or other hardship or are otherwise required to forego a planned transaction because of the restrictions imposed by this Policy. Personal financial emergency or other personal circumstances will not excuse a failure to comply with this Policy.

3. Insider Trading and Tipping

Insider Trading and Tipping. Directors, officers, employees and other individuals who possess material nonpublic information are prohibited from the following illegal activities, which are commonly referred to as "***insider trading***":

- Trading "on the basis" of material nonpublic information (i.e., as long as they are aware of such information). It is not a defense that the person did not "use" the material nonpublic information for purposes of the transaction.

- Disclosing material nonpublic information directly or indirectly to others who then trade based on that information, or making recommendations or expressing opinions on transactions in securities while aware of material nonpublic information (sometimes referred to as "***tipping***"). Both the person who provides the information, recommendation or opinion and the person who trades based on it may be liable.

The U.S. Securities and Exchange Commission (the "***SEC***") and the U.S. Department of Justice pursue insider trading violations vigorously. Cases involving trading through foreign accounts, trading by family members and friends and trading only a small number of shares have been successfully prosecuted. There are no exceptions from insider trading laws or this Policy based on the size of the transaction.

Controlling Person Liability. In addition, a company, as well as individual directors, officers and other supervisory personnel, may be subject to liability as "***controlling persons***" for failure to take appropriate steps to prevent insider trading by those under their supervision, influence or control.

4. Other Prohibited and Problematic Transactions

The types of transactions listed below may expose you and the Company to significant risks. Even if a transaction is not listed below, you are responsible for ensuring that it otherwise complies with the applicable provisions of this Policy, including insider trading and tipping prohibitions, the pre-clearance requirements and procedures applicable to you if you are on the Pre-Clearance List ("***Pre-Clearance Requirements***"), and Trading Blackout Periods.

- *Short Sales.* You are prohibited from making short sales (*i.e.,* the sale of a security that must be borrowed to make delivery) and "selling short against the box" (*i.e.,* a sale with a delayed delivery) with respect to Company securities.

- *Transactions in Derivative Securities.* You are prohibited from engaging in transactions in publicly-traded options, such as puts and calls, and other derivative securities with respect to the Company's securities. This prohibition extends to any hedging or similar transaction designed to decrease the risks associated with holding Company securities. Stock options, stock appreciation rights and other securities issued pursuant to Company benefit plans or other compensatory arrangements with the Company are not subject to this prohibition. Among other reasons for this prohibition, the application of securities laws to derivatives transactions can be complex, and persons engaging in derivatives transactions may subject themselves to an increased risk of violating SEC regulations and other applicable securities laws.

- *Using Company Securities as Collateral for Loans* You are prohibited from pledging Company securities as collateral for loans except to the extent you have made such arrangements prior to the Company's adoption of this Policy. If you default on the loan, the lender may sell the pledged securities as collateral in a foreclosure sale. This sale, even though

not initiated at your request, would be considered a sale for your benefit and, if made at a time when you are aware of material nonpublic information or otherwise are not permitted to trade Company securities, may result in violations of this Policy or securities laws.

- *Holding Company Securities in Margin Accounts* You are prohibited from holding Company securities in margin accounts. Under typical margin arrangements, if you fail to meet a margin call, the broker may be entitled to sell securities held in the margin account without your consent. This sale, even though not initiated at your request, would be considered a sale for your benefit and, if made at a time when you are aware of material nonpublic information or otherwise are not permitted to trade Company securities, may result in violations of this Policy or securities laws.

- *Placing Open Orders with Brokers.* Except in accordance with an approved trading plan, as discussed in Part III (Limited Exceptions), you should exercise caution when placing open orders, such as limit orders or stop orders, with brokers, particularly where the order is likely to remain outstanding for an extended period of time. Open orders may result in the execution of a trade at a time when you are aware of material nonpublic information or otherwise are not permitted to trade in Company securities, which may result in violations of this Policy or securities laws. Additionally, you should so inform any broker with whom you place any open order about the requirements of this Policy at the time any open order is placed.

5. Trading Blackout Periods

To limit the likelihood of trading at times when there is a significant risk of insider trading exposure, the Company has instituted Quarterly Blackout Periods (the "***Trading Blackout Periods***"). The Company may also institute special trading blackout periods from time to time. Whether or not you are subject to a blackout period, you remain subject to the prohibitions on trading on the basis of material nonpublic information and any other applicable restrictions in this Policy. There are no unconditional "safe harbors" for trades made at particular times, and you should exercise good judgment at all times. Even when a trading window is open you may be restricted from trading if you possess material nonpublic information or are otherwise restricted by this Policy.

Quarterly Blackout Periods. Except as discussed in Part III (Limited Exceptions), all employees, consultants, contractors and other agents of the Company are subject to and must refrain from conducting transactions involving the Company's securities during the Quarterly Blackout Periods. Quarterly Blackout Periods start on the last day of the last month of the quarter and ending 24 hours following the release of the Company's earnings for that quarter.

Interim Earnings Guidance Blackout The Company may on occasion issue interim earnings guidance or other potentially material information by means of a press release, SEC filing on Form 8-K or other means designed to achieve widespread dissemination of the information. All employees, consultants, contractors and other agents of the Company should anticipate that trading will be blacked out while the Company is in the process of assembling the information to be released and until the information has been released and fully absorbed by the market.

Special Blackout Periods. From time to time, the Company may also prohibit you from engaging in transactions involving the Company's securities when, in the judgment of the Compliance Officer, a trading blackout is warranted. The Company will generally impose special blackout periods when there are material developments known to the Company that have not yet been disclosed to the public. However, special blackout periods may be declared for any reason. The Company will notify those persons subject to a special blackout period. Each person who has been notified that they are subject to a special blackout period may

not engage in any transaction involving the Company's securities until instructed otherwise by the Compliance Officer, and should not disclose to others the fact of such suspension of trading.

Regulation BTR Blackouts Directors and officers may also be subject to trading blackouts pursuant to Regulation Blackout Trading Restriction ("***Regulation BTR***"), under U.S. federal securities laws. In general, Regulation BTR prohibits any director or officer from engaging in certain transactions involving Company securities during periods when 401(k) plan participants are prevented from purchasing, selling or otherwise acquiring or transferring an interest in certain securities held in individual account plans. Any profits realized from a transaction that violates Regulation BTR are recoverable by the Company, regardless of the intentions of the director or officer effecting the transaction. In addition, individuals who engage in such transactions are subject to sanction by the SEC as well as potential criminal liability. The Company will notify directors and officers if they are subject to a blackout trading restriction under Regulation BTR. Failure to comply with an applicable trading blackout in accordance with Regulation BTR is a violation of law and this Policy.

6. Pre-Clearance of Trades

Except as discussed in Part III (Limited Exceptions), all employees, consultants, contractors and other agents of the Company must refrain from engaging in any transaction involving the Company's securities without first obtaining pre-clearance of the transaction from the Compliance Officer. The Compliance Officer may not engage in a transaction involving the Company's securities unless the Chief Executive Officer has pre-cleared the transaction.

Pre-clearance of a trade, however, is not a defense to a claim of insider trading and does not excuse you from otherwise complying with insider trading laws or this Policy. Further, pre-clearance of a transaction does not constitute an affirmation by the Company or the Compliance Officer that you are not in possession of material nonpublic information. The Compliance Officer is under no obligation to approve a transaction submitted for pre-clearance, and may determine not to permit the transaction.

7. Consequences for Violations

Civil and Criminal Penalties. Potential penalties for insider trading violations, tipping or controlling person liability include criminal and civil fines and penalties, imprisonment and other consequences.

Company Disciplinary Actions. If the Company has a reasonable basis to conclude that you have failed to comply with this Policy, you may be subject to disciplinary action, up to and including dismissal for cause, regardless of whether or not your actions result in a violation of law. It is not necessary for the Company to wait for the filing or conclusion of any civil or criminal action against you before taking disciplinary action. In addition, the Company may give stop transfer and other instructions to its transfer agent with respect to transactions that the Company considers to be in contravention of this Policy.

8. Personal Responsibility

The ultimate responsibility for complying with this Policy and applicable laws and regulations rests with you. You should use your best judgment at all times and consult with your personal legal and financial advisors, as needed. We advise you to seek assistance if you have any questions at all. The rules relating to insider trading can be complex, and a violation of insider trading laws can carry severe consequences.

You should be alert to possible violations and promptly report violations or suspected violations of this Policy to the Compliance Officer (as defined below). If your situation requires that your identity be kept secret, your anonymity will be preserved to the greatest extent reasonably possible. If you wish to remain

anonymous, send a letter addressed to the Compliance Officer at 4800 Montgomery Lane, Suite 220, Bethesda, Maryland, 20814. If you make an anonymous report, please provide as much detail as possible, including any evidence that you believe may be relevant to the issue.

9. Compliance Officer

Please direct any questions, requests or reports as to any of the matters discussed in this Policy to the Principal Financial Officer of the Company (the "***Compliance Officer***"). The Compliance Officer is generally responsible for the administration of this Policy. The Compliance Officer may select others to assist with the execution of his or her duties.

10. Additional Information

Delivery of Policy. This Policy will be delivered to all directors, officers, employees and agents of the Company when they commence service with the Company. In addition, this Policy (or a summary of this Policy) will be circulated periodically. Each director, officer, employee and agent of the Company is required to acknowledge that he or she understands, and agrees to comply with, this Policy.

Amendments. We are committed to continuously reviewing and updating our policies and procedures. The Company therefore reserves the right to amend, alter or terminate this Policy at any time and for any reason, subject to applicable law. A current copy of the Company's policies regarding insider trading may be obtained by contacting the Compliance Officer.

* * *

Nothing in this Insider Trading Policy creates or implies an employment contract or term of employment or a right to continued employment.

The policies in this Insider Trading Policy do not constitute a complete list of Company policies or a complete list of the types of conduct that can result in discipline, up to and including discharge.

PART II

MATERIAL NONPUBLIC INFORMATION: WHAT IT IS AND YOUR OBLIGATIONS

1. Definitions

"Material" Information. Information should be regarded as material if there is a substantial likelihood a reasonable investor would consider it important in deciding whether to buy, hold or sell securities or would view the information as significantly altering the total mix of information in the marketplace about the issuer of the security. Any information that could reasonably be expected to affect the market price of a security is likely to be material, regardless of whether it is positive or negative.

It is not possible to define all categories of "material" information. However, some examples of information that would often be regarded as material include information with respect to:

- Financial results, key metrics, financial condition, earnings pre-announcements, guidance, projections or forecasts, particularly if inconsistent with the Company's guidance or the expectations of the investment community;
- Restatements of financial results, or material impairments, write-offs or restructurings;
- Changes in independent auditors, or notification that the Company may no longer rely on an audit report;
- Business plans or budgets;
- Creation of significant financial obligations, or any significant default under or acceleration of any financial obligation;
- Impending bankruptcy or financial liquidity problems;
- Significant developments involving business relationships, including execution, modification or termination of significant agreements or orders with customers, suppliers, distributors, manufacturers or other business partners;
- Significant information relating to the operation of a product or service, such as new products or services, major modifications or performance issues, defects or recalls, significant pricing changes or other announcements of a significant nature;
- Significant developments in research and development or relating to intellectual property;
- Significant legal or regulatory developments, whether positive or negative, actual or threatened, including litigation or resolving litigation;
- Major events involving the Company's securities, including calls of securities for redemption, adoption of stock repurchase programs, option repricings, stock splits, changes in dividend policies, public or private securities offerings, modification to the rights of security holders or notice of delisting;

- Significant corporate events, such as a pending or proposed merger, joint venture or tender offer, a significant investment, the acquisition or disposition of a significant business or asset or a change in control;
- Major personnel changes, such as changes in senior management or lay-offs;
- Data breaches or other cybersecurity events;
- Updates regarding any prior material disclosure that has materially changed;
- The existence of a special blackout period; and
- Significant disruptions in the Company's operations or loss, potential loss, breach or unauthorized access of the Company's property or assets, including the Company's facilities and information technology infrastructure.

If you have any questions as to whether information should be considered "material," you should consult with the Compliance Officer. In general, it is advisable to resolve any close questions as to the materiality of any information by assuming that the information is material.

"Nonpublic" Information. Generally, information is considered nonpublic if the information has not been broadly disseminated to the public for a sufficient period to be reflected in the price of the security. As a general rule, information should be considered nonpublic until at least two ***full trading days*** have elapsed after the information is broadly distributed to the public in a press release, a public filing with the SEC, a pre-announced public webcast or another broad, non-exclusionary form of public communication. However, depending upon the form of the announcement and the nature of the information, it is possible that information may not be fully absorbed by the marketplace until a later time. Any questions as to whether information is nonpublic should be directed to the Compliance Officer.

The term "***trading day***" means a day on which national stock exchanges and the National Association of Securities Dealers, Inc. Automated Quotation System are open for trading. A "***full***" trading day has elapsed when, after the public disclosure, trading in the relevant security has opened and then closed.

2. Confidentiality of Nonpublic Information

This Policy also prohibits the unauthorized use or disclosure of nonpublic information relating to the Company or other companies, including the Company's distributors, vendors, customers, collaborators, suppliers and competitors. All nonpublic information you acquire in the course of your service with the Company may only be used for legitimate Company business purposes. In addition, nonpublic information of others should be handled in accordance with the terms of any relevant nondisclosure agreements, and the use of any such nonpublic information should be limited to the purpose for which it was disclosed.

You must use all reasonable efforts to safeguard nonpublic information in the Company's possession. You may not disclose nonpublic information about the Company or any other company, unless required by law, or unless (i) disclosure is required for legitimate Company business purposes, (ii) you are authorized to disclose the information and (iii) appropriate steps have been taken to prevent misuse of that information (including entering an appropriate nondisclosure agreement that restricts the disclosure and use of the information, if applicable). This restriction also applies to internal communications within the Company and to communications with agents of the Company. In cases where disclosing nonpublic information to third parties is required, you should coordinate with the Compliance Officer.

3. No Trading on Material Nonpublic Information

Except as discussed in in Part III (Limited Exceptions), you may not, directly or indirectly through others, engage in any transaction involving the Company's securities while aware of material nonpublic information relating to the Company. It is not an excuse that you did not "use" the information in your transaction.

Similarly, you may not engage in transactions involving the securities of any other company if you are aware of material nonpublic information affecting that company, except to the extent the transactions are analogous to those described in Part III (Limited Exceptions). For example, you may be involved in a proposed transaction involving a prospective business relationship or transaction with another company. If information about that transaction constitutes material nonpublic information for that other company, you would be prohibited from engaging in transactions involving the securities of that other company (as well as transactions involving Company securities, if that information is material to the Company). Additionally, information need not directly involve a company to be considered material to it. For example, news of a merger between two companies may be material to an, otherwise uninvolved, third company in the same sector or industry. It is important to note that "materiality" is different for different companies. Information that is not material to the Company may be material to another company.

4. No Disclosing Material Nonpublic Information for the Benefit of Others

You may not disclose material nonpublic information concerning the Company or any other company to friends, family members or any other person or entity not authorized to receive such information where such person or entity may benefit by trading on the basis of such information. In addition, you may not make recommendations or express opinions on the basis of material nonpublic information as to trading in the securities of companies to which such information relates. You are prohibited from engaging in these actions whether or not you derive any profit or personal benefit from doing so. The prohibition against disclosure of material nonpublic information includes disclosure (even anonymous disclosure) via the internet, blogs, investor forums or chat rooms where companies and their prospects are discussed.

5. Obligation to Disclose Material Nonpublic Information to the Company

You may not enter into any transaction, whether or not it is described in Part III (Limited Exceptions), unless you have disclosed any material nonpublic information that you become aware of in the course of your service with the Company, and that senior management is not aware of, to the Compliance Officer. If you are a member of senior management, the information must be disclosed to the Chief Executive Officer, and if you are the Chief Executive Officer or a director, you must disclose the information to the Company's board of directors ("***Board of Directors***"), before any transaction is permissible.

6. Responding to Outside Inquiries for Information

If you receive an inquiry from someone outside of the Company, such as a stock analyst, for information, you should refer the inquiry to the Principal Financial Officer of the Chief Executive Officer. The Company is required under Regulation FD (Fair Disclosure) of the U.S. federal securities laws to avoid the selective disclosure of material nonpublic information. In general, the regulation provides that when a public company discloses material nonpublic information, it must provide broad, non-exclusionary access to the information. Violations of this regulation can subject the Company to SEC enforcement actions, which may result in injunctions and severe monetary penalties. The Company has established procedures for releasing material information in a manner that is designed to achieve broad public dissemination of the

information immediately upon its release in compliance with applicable law. Please consult the Company's Regulation FD Policy for more details.

7. Protected Activity Not Prohibited

Nothing in this Policy, or any related guidelines or other documents or information provided in connection with this Policy, shall in any way limit or prohibit you from engaging in any of the protected activities set forth in the Company's Whistleblower Policy, as amended from time to time.

PART III

LIMITED EXCEPTIONS TO THIS POLICY

The following are certain limited exceptions to the restrictions imposed by the Company under this Policy. Please be aware that even if a transaction is subject to an exception to this Policy, you will need to separately assess whether the transaction complies with applicable law (for example, "short-swing" trading restrictions under Section 16, to the extent applicable). You are responsible for complying with applicable law at all times.

1. Transactions Pursuant to a Trading Plan that Complies with SEC Rules

The SEC has enacted rules that provide an affirmative defense against alleged violations of U.S. federal insider trading laws for transactions pursuant to trading plans that meet certain requirements. In general, these rules, as set forth in Rule 10b5-1 under the Securities Exchange Act of 1934, as amended, provide for an affirmative defense if you enter into a contract, provide instructions or adopt a written plan for trading securities when you are not aware of material nonpublic information. The contract, instructions or plan must (i) specify the amount, price and date of the transaction, (ii) specify an objective method for determining the amount, price and date of the transaction and/or (iii) place any subsequent discretion for determining the amount, price and date of the transaction in another person who is not, at the time of the transaction, aware of material nonpublic information.

Transactions made pursuant to a written trading plan that (i) complies with the affirmative defense set forth in Rule 10b5-1 and (ii) is approved by the Compliance Officer are not subject to the restrictions in this Policy against trades made while aware of material nonpublic information or to the blackout periods established under this Policy. In approving a trading plan, the Compliance Officer may, in furtherance of the objectives expressed in this Policy, impose criteria in addition to those set forth in Rule 10b5-1. You should therefore confer with the Compliance Officer prior to entering into any trading plan.

The SEC rules regarding trading plans are complex and must be complied with completely to be effective. The description provided above is only a summary, and the Company strongly advises that you consult with your legal advisor if you intend to adopt a trading plan. While trading plans are subject to review and approval by the Company, the individual adopting the trading plan is ultimately responsible for compliance with Rule 10b5-1 and ensuring that the trading plan complies with this Policy.

The Company will determine which individuals may use trading plans. Trading plans must (1) be filed with the Compliance Officer, (2) comply with the requirements of Rule 10b5-1, and (3) be accompanied with an executed certificate stating that the trading plan complies with Rule 10b5-1 and any other criteria established by the Company. If the Compliance Officer is the requester, then the Company's Chief Executive Officer must approve the written 10b5-1 trading plan. The Company may publicly disclose information regarding trading plans.

2. Receipt and Vesting of Stock Options, Restricted Stock, Restricted Stock Units and Stock Appreciation Rights

The trading restrictions under this Policy do not apply to the acceptance or purchase of stock options, restricted stock, restricted stock units or stock appreciation rights issued or offered by the Company. The trading restrictions under this Policy also do not apply to the vesting, cancellation or forfeiture of stock options, restricted stock, restricted stock units or stock appreciation rights in accordance with applicable plans and agreements.

3. Exercise of Stock Options for Cash; Net Share Withholding

The trading restrictions under this Policy do not apply to the exercise of stock options for cash under the Company's equity incentive plans. Likewise, the trading restrictions under this Policy do not apply to the exercise of stock options in a stock-for-stock exercise with the Company or an election to have the Company withhold securities to cover tax obligations in connection with an option exercise (x) as required by either the Company's board of directors (or a committee thereof) or the award agreement governing such equity award or (y) as you elect, if permitted by the Company, so long as that election is irrevocable and made in writing at a time when a trading blackout is not in place and the individual is not in possession of material nonpublic information. However, the trading restrictions under this Policy do apply to (i) the sale of any securities issued upon the exercise of a stock option, (ii) a cashless exercise of a stock option through a broker, since this involves selling a portion of the underlying shares to cover the costs of exercise, and (iii) any other market sale for the purpose of generating the cash needed to pay the exercise price of an option.

4. Purchases from an Employee Stock Purchase Plan

The trading restrictions in this Policy do not apply to elections with respect to participation in any employee stock purchase plan that the Company adopts or to purchases of securities under such plan. However, the trading restrictions do apply to any subsequent sales of any such securities.

5. Certain 401(k) Plan Transactions

The trading restrictions in this Policy do not apply to purchases of Company stock in a 401(k) plan resulting from periodic contributions to the plan based on your payroll contribution election. The trading restrictions do apply, however, to elections you make under the 401(k) plan to (i) increase or decrease the amount of your contributions under a 401(k) plan, if such increase or decrease will increase or decrease the amount of your contributions that will be allocated to a Company stock fund (ii) increase or decrease the percentage of your contributions that will be allocated to a Company stock fund, (iii) move balances into or out of a Company stock fund, (iv) borrow money against a 401(k) plan account if the loan will result in liquidation of some or all of your Company stock fund balance, and (v) pre-pay a plan loan if the pre-payment will result in the allocation of loan proceeds to a Company stock fund.

6. Certain Transfers by Will and for Tax Planning Purposes

The trading restrictions in this Policy do not apply to transfers by will or the laws of descent or distribution and, provided that prior written notice is provided to the Compliance Officer, distributions or transfers (such as certain tax planning or estate planning transfers) that effect only a change in the form of beneficial interest without changing your pecuniary interest in the Company's securities.

7. Other Exceptions

Stock splits, stock dividends and similar transactions. The trading restrictions under this Policy do not apply to a change in the number of securities held as a result of a stock split or stock dividend applying equally to all securities of a class, or similar transactions.

Change in form of ownership. Transactions that involve merely a change in the form in which you own securities are permissible. For example, you may transfer shares to an *inter vivos* trust of which you are the sole beneficiary during your lifetime.

Other. Any other exception from this Policy must be approved by the Compliance Officer, in consultation with the Board of Directors or an independent committee of the Board of Directors.

PART IV

COMPLIANCE WITH SECTION 16
OF THE SECURITIES EXCHANGE ACT

1. Obligations Under Section 16

Section 16 and the related rules and regulations set forth (i) reporting obligations, (ii) limitations on "short-swing" transactions and (iii) limitations on short sales and other transactions applicable to directors, officers, large shareholders and certain other persons. The Company has provided, or will provide, memoranda and other materials addressing these matters.

The Company has designated certain persons as being required to comply with Section 16 and the related rules and regulations because of their positions with the Company. The persons subject to Section 16 may change from time to time as appropriate to reflect the election of new officers or directors, any change in the responsibilities of officers or other employees and any promotions, demotions, resignations or departures.

Even if you are not designated by the Company as being required to comply with Section 16, you may be subject to Section 16 reporting obligations because of, for example, your shareholdings.

2. Notification Requirements to Facilitate Section 16 Reporting

To facilitate timely reporting of transactions pursuant to Section 16 requirements, each person subject to Section 16 reporting requirements must provide, or must ensure that his or her broker provides, the Company with detailed information (e.g., trade date, number of shares, exact price, etc.) regarding his or her transactions involving the Company's securities, including gifts, transfers, pledges and transactions pursuant to a trading plan, both prior to (to confirm compliance with pre-clearance procedures, if applicable) and promptly following execution.

3. Personal Responsibility Under Section 16

The obligation to file Section 16 reports, and to otherwise comply with Section 16, is personal. The Company is not responsible for the failure to comply with Section 16 requirements.

Subsidiaries of Gain Therapeutics, Inc.

Name of Subsidiary	**Jurisdiction of Incorporation**
GT Gain Therapeutics SA	Switzerland
Gain Therapeutics Australia Pty Ltd	Australia

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

1. Registration Statement (Form S-8 No. 333-282205) pertaining to the Gain Therapeutics, Inc. 2022 Equity Incentive Plan,
2. Registration Statement (Form S-8 No. 333-255061) pertaining to the Gain Therapeutics, Inc. 2020 Omnibus Incentive Plan,
3. Registration Statement (Form S-8 No. 333-266142) pertaining to the Gain Therapeutics, Inc. 2021 Inducement Equity Incentive Plan and the Gain Therapeutics, Inc. 2022 Equity Incentive Plan,
4. Registration Statement (Form S-8 No. 333-272255) pertaining to the Gain Therapeutics, Inc. 2022 Equity Incentive Plan,
5. Registration Statement (Form S-3 No. 333-265061) of Gain Therapeutics, Inc., and
6. Registration Statement (Form S-1 No. 333-276145) of Gain Therapeutics, Inc.;

of our report dated March 27, 2025, with respect to the consolidated financial statements of Gain Therapeutics, Inc., included in this Annual Report (Form 10-K) for the year ended December 31, 2024.

/s/ Ernst & Young AG

Lugano, Switzerland

March 27, 2025

Exhibit 31.1

Management Certification Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

I, Gene Mack, certify that:

1. I have reviewed this Annual Report on Form 10-K for the fiscal year ended December 31, 2024 of Gain Therapeutics, Inc.;

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

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

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

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

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

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

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

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

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

March 27, 2025
Date

/s/ Gene Mack
Gene Mack
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

Management Certification Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002

I, Gianluca Fuggetta, certify that:

1. I have reviewed this Annual Report on Form 10-K for the fiscal year ended December 31, 2024 of Gain Therapeutics, Inc.;

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

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

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

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

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

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

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

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

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

March 27, 2025
Date

/s/ Gianluca Fuggetta
Gianluca Fuggetta
Senior Vice President Finance
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

Certification of CEO and PFO Pursuant to
18 U.S.C. Section 1350,
as Adopted Pursuant to
Section 906 of the Sarbanes-Oxley Act of 2002

In connection with the Annual Report on Form 10-K of Gain Therapeutics, Inc. (the "Company") for the year ended December 31, 2024 to which this certification is attached as Exhibit 32.1 (the "Report"), Gene Mack, as Chief Executive Officer of the Company, and Gianluca Fuggetta, as Principal Financial Officer of the Company, each hereby certifies, pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), that, to the best of his knowledge:

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

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

Date: March 27, 2025

/s/ Gene Mack
Gene Mack
Chief Executive Officer
(Principal Executive Officer)

/s/ Gianluca Fuggetta
Gianluca Fuggetta
Senior Vice President Finance
(Principal Financial Officer and Principal Accounting Officer)

This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Gain Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.