UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the fiscal year ended December 31, 2025

OR

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

Commission File Number: 001-39979

VOR BIOPHARMA INC.

(Exact name of registrant as specified in its charter)

Delaware	**81-1591163**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
500 Boylston Street	
Suite 1350	
Boston, Massachusetts	**02116**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 655-6580

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

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

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

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

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

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

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

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

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

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

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

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

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

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

As of June 30, 2025, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the registrant's common stock, $0.0001 par value per share ("Common Stock"), held by non-affiliates of the registrant was approximately $76.1 million based upon the closing price of the Common Stock on June 30, 2025.

The number of shares of the registrant's Common Stock outstanding as of March 23, 2026 was 48,847,504.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for its 2026 Annual Meeting of Stockholders, to be filed with the Securities and Exchange Commission no later than 120 days after December 31, 2025, are incorporated by reference in Part III of this Annual Report on Form 10-K.

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Note Regarding Company References

Throughout this Annual Report on Form 10-K ("Annual Report"), the "Company," "Vor," "Vor Bio," "Vor Biopharma Inc.," "we," "us," and "our," except where the context requires otherwise, refer to Vor Biopharma Inc. and its consolidated subsidiary, and "our board of directors" refers to the board of directors of Vor Biopharma Inc.

Special Note Regarding Forward-Looking Statements and Industry Data

This Annual Report contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, and objectives of management, are forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "would," "expect," "plan," "anticipate," "could," "might," "intend," "target," "ongoing," "project," "estimate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions intended to identify statements about the future. These statements speak only as of the date of this Annual Report and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by the forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements include, without limitation, statements about:

- the timing, progress and results of our clinical trials of our product candidate and clinical trials for any future product candidates, including statements regarding the timing and pace of initiation, enrollment and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and plans with respect to our research and development programs;
- the timing of any submission of filings for regulatory approval of, and our ability to obtain and maintain regulatory approvals for, our product candidate and any future product candidates for any indication;
- our ability to identify patients with the diseases treated by our product candidate and any future product candidates, and to enroll patients in clinical trials;
- our expectations regarding the market acceptance and opportunity for and clinical utility of our product candidate and any future product candidates, if approved for commercial use;
- our expectations regarding the scope of any approved indication for any product candidate;
- our ability to successfully commercialize our product candidate or any future product candidates;
- our estimates of our expenses, ongoing losses, future revenue and capital requirements and our need for or ability to obtain additional funding;
- our ability to establish or maintain collaborations or strategic relationships;
- our ability to identify, recruit and retain key personnel, including executive officers and members of management;
- our reliance upon intellectual property licensed from third parties and our ability to obtain such licenses on commercially reasonable terms or at all;
- our ability to protect and enforce our intellectual property position for our product candidate or any future product candidates, and the scope of such protection;
- our financial performance;
- the period over which we estimate our existing cash, cash equivalents and marketable securities will be sufficient to fund our future operating expenses and capital expenditure requirements;
- our expectations regarding the closing of the 2026 Private Placement;

- our competitive position and the development of and projections relating to our competition or our industry; and
- the impact of laws and regulations.

You should read this Annual Report and the documents that we have filed as exhibits to this Annual Report completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Annual Report are made as of the date of this Annual Report, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law. We have included important factors in this Annual Report, particularly in the "Summary Risk Factors" and "Risk Factors" sections, that could cause actual results or events to differ materially from the forward-looking statements that we make.

This Annual Report includes statistical and other industry and market data, which we obtained from our own internal estimates and research, as well as from industry and general publications and research, surveys, and studies conducted by third parties. Industry publications, studies, and surveys generally state that they have been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe that each of these studies and publications is reliable, we have not independently verified market and industry data from third-party sources. While we believe our internal company research is reliable and the market definitions are appropriate, neither such research nor these definitions have been verified by any independent source.

All brand names or trademarks appearing in this Annual Report are the property of their respective owners.

Summary Risk Factors

Our business is subject to a number of risks that if realized could materially affect our business, financial condition, results of operations, cash flows and access to liquidity. These risks are discussed more fully in the "Risk Factors" section of this Annual Report. Our principal risks include the following:

- We have incurred significant net losses since inception. We expect to incur net losses for the foreseeable future and may never achieve or maintain profitability.
- We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce or eliminate our research and product development programs or future commercialization efforts.
- We have a limited operating history, have not yet completed any clinical trials and have no history of commercializing products, which may make it difficult to evaluate the success of our business to date and to assess our future viability.
- We are substantially dependent on the success of our lead product candidate, telitacicept. If we are unable to complete development of, obtain approval for and commercialize telitacicept in a timely manner, our business will be harmed.
- We may derive results and data for telitacicept from clinical trials conducted by RemeGen in China; our access to the clinical results and data may be limited and there is no assurance that the clinical data from any such trials will be accepted or considered by the FDA, or other comparable regulatory authorities.
- We are dependent on third parties accurately generating and reporting data related to our product candidate, and their conduct could adversely affect our business.
- We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize our product candidates, if approved.

- Success in preclinical studies or clinical trials may not be indicative of results in future clinical trials, particularly for clinical trials that involve only a small number of patients.
- If we experience significant delays or difficulties in the enrollment or retention of patients in clinical trials, the cost of developing product candidates could increase and our receipt of necessary regulatory approvals could be delayed or prevented.
- We will continue to contract with third parties for the manufacture and supply of materials for development of our product candidates, advancement of our current and future clinical trials, and potential commercialization of our product candidates. This increases the risk that we will not have sufficient quantities and quality of such materials for the development of our product candidates, or that such supply will not be available to us at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.
- We are highly dependent on intellectual property licensed from third parties and termination of these licenses could result in the loss of significant rights, which would harm our business.
- Third-party claims of intellectual property infringement, misappropriation or other violations may prevent or delay our product discovery and development efforts and have a material adverse effect on our business.

PART I

Item 1. Business.

Overview

Vor Biopharma, Inc. ("Vor Bio") is a clinical-stage biopharmaceutical company focused on developing a novel therapy in the treatment of autoimmune diseases. In June 2025, we in-licensed telitacicept from RemeGen Co., Ltd. (“RemeGen”). Pursuant to our license agreement with RemeGen, we were granted an exclusive license to develop and commercialize telitacicept outside of the Greater China region, which includes mainland China, Hong Kong, Macau and Taiwan. RemeGen retains development and commercialization rights in Greater China.

Telitacicept is approved in China for the treatment of systemic lupus erythematosus (“SLE”), rheumatoid arthritis (“RA”), and generalized myasthenia gravis (“gMG”), and has two Biologics License Applications ("BLAs") filed and pending in China for the treatment of Sjögren’s disease (“SjD”) and IgA nephrapathy (“IgAN”). Our global Phase 3 clinical trial in gMG is currently underway across the United States, Europe, South America, and Asia to support potential approval in the United States, Europe, and Japan. We have recently initiated a global Phase 3 clinical trial in SjD, with the first patient dosing in March 2026, and anticipate enrolling at clinical sites across the United States, Europe, South America, and Asia to support potential approval in the United States, Europe, and Japan.

Telitacicept is a novel fusion protein for treating autoimmune diseases. It is constructed with the extracellular domain of the human transmembrane activator and calcium modulator and cyclophilin ligand interactor (“TACI”) receptor and the fragment crystallizable (“Fc”) domain of human immunoglobulin G (“IgG”). Telitacicept targets and acts on two cell signaling molecules critical for B lymphocyte development: B cell lymphocyte stimulator (“BLyS”), also known as B cell activating factor (“BAFF”), and a proliferation inducing ligand (“APRIL”), which allows it to effectively reduce B cell mediated autoimmune responses that are implicated in several autoimmune diseases.

Our Strategy

Vor Bio is executing a focused strategy to become a leading global company in autoimmune therapeutics. Our strategic priorities include:

1. **Rapid Global Development of Telitacicept:** We are advancing telitacicept through global Phase 3 trials, with the near-term goal of regulatory approval in the United States, Europe, and Japan for gMG and SjD. Our strategy leverages prior trials of telitacicept conducted in China by RemeGen.

2. **Pipeline Expansion:** Telitacicept’s dual inhibition of BAFF/APRIL provides a platform to address a broad range of B cell-driven autoimmune diseases. We are evaluating opportunities to develop telitacicept in additional indications based on scientific rationale and unmet market need.

3. **Global Commercial Preparedness:** With telitacicept already approved in China for SLE, RA and gMG and a registrational program underway in major global markets, we are building commercial capabilities and infrastructure to support potential launches.

Our Autoimmune Programs

Generalized Myasthenia Gravis

gMG is a rare, chronic autoimmune neuromuscular disorder characterized by fluctuating skeletal muscle weakness resulting from pathogenic autoantibodies, most frequently the targeting components of the neuromuscular junction. These autoantibodies, about 85% of which are acetylcholine receptor (“AChR”) and 5-8% of which are muscle-specific tyrosine kinase (“MuSK”), impair synaptic transmission, leading to a disabling and potentially life-threatening condition that requires long-term immunomodulation to control disease activity.

gMG has a prevalence of approximately 150 to 250 individuals per million worldwide, with a rising incidence among aging populations. In the United States, based on a 2021 claims analysis, the prevalence is thought to be comparable to Europe at approximately 370 individuals per million, and it is estimated that approximately 100,000 individuals have MG in the United States.

The treatment goals for gMG include achieving minimal symptoms while minimizing treatment side effects. Even with newer treatments, MG poses significant physical, psychological, and economic impacts despite available treatments, with potential for exacerbations, myasthenia crises which could be life threatening, and complications such as infections or autoimmune comorbidities. Drug therapy for gMG typically begins with acetylcholinesterase inhibitors (such as pyridostigmine). However, as acetylcholinesterase inhibitors may improve symptoms, they are often insufficient. Traditional immunosuppressive therapies, such as corticosteroids, azathioprine, and mycophenolate mofetil, remained first-line treatments for decades, despite their delayed onset of action, cumulative toxicity, and inconsistent disease control. Recent biologics have introduced targeted immune modulation with improved clinical outcomes. Currently there are two classes of biologics in clinical development that have already obtained approval: neonatal fragment crystallizable receptor ("FcRn") antagonists and complement inhibitors.

FcRn antagonists enhance immunoglobulin G ("IgG") catabolism and have demonstrated clinical benefit in AChR antibody positive patients. They are administered in cycles of four weeks or six weeks due to the deep reduction in IgG level, which increases the potential for severe infection. Complement inhibitors block terminal complement activation and have shown efficacy in reducing disease severity. However, complement inhibitors carry a black box warning on the label, which requires a Risk Evaluation and Mitigation Strategies ("REMS") program requiring patients to be vaccinated to prevent serious brain infection prior to initiating the therapy. Most recently, a monoclonal antibody targeting CD19, depleting a broad range of B-cells, showed clinical benefit in Phase 3 clinical trials.

However, none of these agents directly modulates the cytokine environment that sustains autoreactive B cells and plasma cells. B cell maturation and plasma cell survival are critically regulated by two cytokines of the TNF ligand superfamily: BAFF and APRIL. Elevated circulating levels of BAFF and APRIL have been detected in autoimmune diseases, including gMG. In gMG specifically, BAFF has been shown to be elevated in both serum and thymic tissues of patients with active disease and correlates with higher anti-AChR antibody titers.

Phase 3 Clinical Trial in patients with gMG in China

Telitacicept was evaluated by RemeGen in a Phase 3 clinical trial in patients with gMG in China. The trial enrolled 114 patients and consisted of a double-blind treatment period ("Part A") and an open-label treatment period ("Part B"). In Part A, patients were randomized 1:1 to receive either subcutaneous telitacicept 240 mg or placebo once a week for 24 doses. After completing Part A, patients were automatically entered into Part B, in which all patients receive weekly subcutaneous telitacicept 240 mg for 24 weeks.

The primary endpoint of the trial was change from baseline in the Myasthenia Gravis Activities of Daily Living ("MG-ADL") score at week 24 of Part A. MG-ADL is an 8-item patient-reported scale that measures MG symptoms and functional status. Each item ranges from 0-3 for a total score range of 0-24. The MG-ADL scale quantifies the impact of MG symptoms on daily life quality, focusing on patients' subjective experience and daily function. A secondary endpoint was change from baseline in the Quantitative Myasthenia Gravis ("QMG") score, which is a 13-item scale used to quantify disease severity in MG, with total QMG scores ranging from 0-39. The QMG score assesses the strength and endurance of the whole body muscle group, focusing on objective measurement. There is a strong correlation between MG-ADL and QMG in evaluating treatment response, and the combination of MG-ADL and QMG can comprehensively reflect the disease severity.

The 24-week data from Part A of the Phase 3 trial were presented at the 2025 American Academy of Neurology ("AAN") meeting in April 2025 and in European Academy of Neurology in June 2025. The 48-week data from Part B of the Phase 3 trial were presented at the American Association of Neuromuscular & Electrodiagnostic Medicine ("AANEM") Annual Meeting in October 2025. The data at week 48 demonstrated:

- Patients on telitacicept throughout the trial achieved a -7.5 mean MG-ADL change from baseline, while placebo crossover patients achieved -6.3; 96.2% of continuous patients and 90.2% of crossover patients reached ≥3-point improvement.

- Patients on telitacicept throughout the trial achieved a -9.8 mean QMG change, while placebo crossover patients achieved -9.3; 94.2% of continuous patients and 90.2% of crossover patients reached ≥5-point improvement.
- Telitacicept demonstrated a favorable safety profile comparable to placebo and consistent with prior clinical trials and post-marketing data across other autoimmune indications, including SLE, RA, SjD, and IgAN. No new safety signals were observed. Most adverse events were mild to moderate in severity.
- No injection site reactions were reported during the open label extension period in patients previously on telitacicept. Injection site reactions in placebo crossover patients were mild, self-resolving, and did not lead to discontinuation.

Global Phase 3 Clinical Trial in patients with gMG

Telitacicept is currently being evaluated in a global Phase 3 clinical trial, for which we have assumed responsibility from RemeGen in connection with the license agreement, for the treatment of gMG. The trial is currently recruiting in North America, Europe, Latin America and Asia to support potential approval in the United States, Europe and Japan. In July 2024, the clinical trial enrolled a patient in the United States, the first in the global clinical trial. The trial is a randomized, double-blind, placebo controlled trial with an open-label extension period. The primary endpoint is change from baseline in MG-ADL at week 24. Key secondary endpoints include change from baseline in QMG score, proportion of patients achieving MG-ADL score reduction of at least 2 points, proportion of patients achieving QMG score reduction of at least 3 points at week 24 and change from baseline in MG Quality of Life (“MG-QOL15r”) scale at week 24. The MG-QoL15r is a 15-item patient-reported outcome measure designed to assess quality of life in patients with MG. Each item in the scale is scored on a 0-2 point scale, with the total score ranging from 0 to 30. Higher scores indicate a more severe impact of the disease on aspects of the patient's life. A decrease from baseline score indicates improvement.

Topline data from the trial is anticipated in the first half of 2027.

Sjögren’s Disease

SjD is a chronic, systemic autoimmune disorder characterized by lymphocytic infiltration and progressive dysfunction of exocrine glands, most notably the salivary and lacrimal glands, resulting in xerostomia (dry mouth) and keratoconjunctivitis sicca (dry eyes). In addition to glandular involvement, SjD is a heterogeneous, multi-organ disease that may affect the musculoskeletal, pulmonary, renal, neurologic and hematologic systems. The disease is associated with the production of pathogenic autoantibodies, most commonly Anti–Sjögren’s-syndrome-related antigen A ("anti-Ro/SSA") antibodies, and is driven in large part by B-cell hyperactivity and aberrant plasma cell survival. Chronic immune stimulation and persistent B-cell activation increase the risk of serious complications, including non-Hodgkin’s lymphoma.

SjD is one of the most prevalent systemic autoimmune diseases, with an estimated prevalence of more than 300,000 diagnosed patients in the United States alone, and epidemiology broadly comparable to systemic lupus erythematosus. The disease disproportionately affects women, typically in mid-life, and is often underdiagnosed due to heterogeneous presentation and overlap with other autoimmune conditions. Diagnosis is multifactorial and generally requires a combination of serologic markers (including anti-Ro/SSA positivity), objective measures of glandular dysfunction, and systemic disease activity assessments such as the EULAR Sjögren’s Syndrome Disease Activity Index (“ESSDAI”), a clinician-reported instrument that evaluates 12 organ domains to quantify systemic involvement, and EULAR Sjögren’s Syndrome Patient Reported Index (“ESSPRI”), a three-item patient-reported outcome measure designed to assess symptom severity in patients with Sjögren’s disease, including dryness, fatigue and pain.

The treatment goals for SjD include reducing systemic disease activity, preserving glandular function, preventing organ damage and lymphoma, and improving patient-reported symptoms such as dryness, fatigue, and pain. However, there are currently no approved disease-modifying therapies for SjD in the United States. Management is largely symptomatic and supportive, consisting of artificial tears and saliva substitutes for sicca symptoms and off-label use of immunomodulatory agents, including hydroxychloroquine, corticosteroids, mycophenolate mofetil and

rituximab, for patients with systemic manifestations. These approaches are often limited by modest efficacy, delayed onset of action, cumulative toxicity, or inconsistent control of disease activity.

Multiple targeted biologic approaches are in clinical development, primarily focused on B-cell biology. These include monoclonal antibodies targeting BAFF-R, CD40-CD40L signaling, and FcRn. FcRn antagonists reduce circulating immunoglobulin G levels and have demonstrated activity in mid-stage trials; however, they may not address other immunoglobulin isotypes or upstream drivers of B-cell dysregulation. CD40 pathway inhibitors aim to modulate T-cell and B-cell co-stimulation but have shown variable efficacy across studies. Anti-BAFF-R agents seek to reduce survival signals for autoreactive B cells, although earlier single-pathway approaches have yielded mixed clinical results. Most recently, a monoclonal antibody targeting BAFF-R showed relatively modest clinical benefit compared to placebo in Phase 3 clinical trials.

Elevated levels of BAFF and APRIL have been observed in patients with SjD and correlate with disease activity and autoantibody production. Dual inhibition of BAFF and APRIL is designed to reduce survival signals for autoreactive B cells and long-lived plasma cells, thereby decreasing pathogenic autoantibody production while preserving broader immune function. We believe that upstream modulation of both pathways may offer the potential for deeper and more durable disease control compared to approaches that focus solely on IgG reduction or single cytokine blockade.

Phase 3 Clinical Trial in patients with SjD in China

Telitacicept was evaluated by RemeGen in a Phase 3 clinical trial in patients with active SjD in China. The trial enrolled 381 patients and consisted of a 24-week double-blind treatment period ("Stage A") followed by a 24-week double-blind extension period ("Stage B"). In Stage A, patients were randomized 1:1:1 to receive subcutaneous telitacicept 80 mg, telitacicept 160 mg, or placebo once weekly for 24 doses. After completing Stage A, patients automatically entered Stage B and maintained their original blinded treatment assignment, with placebo patients eligible for blinded re-randomization to telitacicept 80 mg or 160 mg if deemed non-responders by investigators.

The primary endpoint of the trial was change from baseline in the ESSDAI score at Week 24 of Stage A. Secondary endpoints included change from baseline in ClinESSDAI, a closely related index focused on clinical features without serologic components, as well as patient-reported outcomes and glandular function measures.

The 48-week data including Stage A and B from the Phase 3 trial were presented at the American College of Rheumatology ("ACR") Annual Meeting in October 2025. The data at week 48 demonstrated:

- Mean change in ESSDAI: At week 24, -4.4 (160mg), -3.0 (80mg), and -0.6 (placebo); at week 48, -4.6 (160mg), -3.2 (80mg), and -0.4 (placebo), demonstrating durable, dose-dependent improvement in systemic disease activity.
- Mean change in ESSPRI: At week 24, -1.88 (160mg), -1.31 (80mg), and -0.36 (placebo); at week 48, -2.56 (160mg), -1.74 (80mg), and -0.41 (placebo), showing sustained symptomatic benefit in dryness, fatigue, and pain.
- ≥3-point ESSDAI improvement: At week 24, 71.8% (160mg), 47.1% (80mg), and 19.3% (placebo); at week 48, 73.0% (160 mg), 49.1% (80mg), and 16.5% (placebo).
- Participants with ESSDAI <5 (low disease activity): At week 24, 49.6% (160mg), 28.8% (80mg), and 10.9% (placebo); at week 48, 55.0% (160mg), 32.7% (80mg), and 12.2% (placebo).
- Participants with ≥1-point or ≥15% ESSPRI reduction: At week 24, 86.2% (160mg), 63.0% (80mg), and 32.2% (placebo); at week 48, 89.1% (160mg), 75.4% (80mg), and 33.3% (placebo).
- Change from baseline in MFI-20 total (fatigue): At weeks 24 and 48, telitacicept 160mg produced a statistically significant and clinically meaningful reduction in fatigue versus 80mg and placebo, with improvements sustained through the open-label extension period.
- Telitacicept demonstrated a favorable safety profile comparable to placebo and consistent with prior studies across other autoimmune indications, including systemic lupus erythematosus, rheumatoid arthritis, myasthenia gravis, and IgA nephropathy. No new safety signals were observed. Most adverse events were mild to moderate in severity.

Global Phase 3 Clinical Trial in patients with SjD

We have recently initiated a global Phase 3 clinical trial evaluating telitacicept for the treatment of SjD, with first patient dosing in March 2026. The trial anticipates recruiting approximately 250 adults with SjD in the United States, Europe, South America, and Asia. The trial is a randomized, double-blind, placebo-controlled trial. The primary endpoint is change from baseline in the ESSDAI score at week 48. Additional endpoints will evaluate the effect of telitacicept at week 48 across other measures of systemic disease activity, glandular function, and patient-reported symptoms, including change from baseline in ESSPRI score, stimulated whole salivary flow, unstimulated whole salivary flow, Schirmer's test, Short Form Health Survey (SF-36) score, Functional Assessment of Chronic Illness Therapy-Fatigue **(**FACIT-F) score, as well as proportion of patients whose ESSDAI score is <5 and ESSPRI score decreased from baseline by ≥ 1 or 15%.

Regulatory Overview

Telitacicept has received Orphan Drug Designation ("ODD") from both the U.S. Food and Drug Administration ("FDA") and European Medicines Agency ("EMA") for the treatment of gMG.

In January 2024, the FDA cleared the Investigational New Drug ("IND") application for the global multi-center Phase 3 clinical trial of telitacicept for the treatment of adult patients with SjD. In March 2024, telitacicept received Fast-Track Designation ("FTD") from the FDA for the treatment of adult patients with SjD.

Discontinued Clinical and Preclinical Development Programs

Prior to our in-license of telitacicept from RemeGen, our efforts were focused on developing therapies to treat blood cancers by genetically engineering hematopoetic stem cells ("HSCs") from healthy donors to remove therapeutic targets and introducing them into patients, thereby creating patients' bone marrow and blood systems that are shielded from on-target toxicities from targeted therapies, and then coupling targeted therapies such as antibody drug conjugates or our own chimeric antigen receptor ("CAR") T cell therapies with these shielded HSC transplants. On May 8, 2025, we announced the winddown of our prior clinical and manufacturing operations. Our discontinued clinical and preclinical development programs include the following:

- Trem-cel: Tremtelectogene empogeditemcel (trem-cel) is a genome-edited hematopoietic stem and progenitor allogeneic donor product candidate where CD33 has been deleted using genome engineering. We were previously evaluating trem-cel in a Phase 1/2a clinical trial (VBP101) in patients with CD33-positive acute myeloid leukemia ("AML") or myelodysplastic syndrome ("MDS") at high risk of relapse.
- VCAR33: VCAR33 is manufactured from lymphocytes collected from the patient's original transplant donor, generating a CAR-T cell therapy that is exactly matched to the recipient's engrafted blood system. We were previously evaluating VCAR33 in a Phase 1/2 clinical trial (VBP301) in patients with relapsed or refractory AML after standard-of-care transplant or a trem-cel transplant.
- Preclinical Programs: Our previous preclinical programs included exploring trem-cel in combination with VCAR33 utilizing cells from the same healthy donor for both trem-cel and VCAR33, which we referred to as the trem-cel+VCAR33 Treatment System; VADC45, an antibody drug conjugate that targets the CD45 protein; and a CD33-CLL1 multiplex-edited HSC therapy in combination with a CD33-CLL1 multi-specific CAR-T therapy, which we referred to as the CD33-CLL1 Treatment System.

In August and September 2025, we sold certain intellectual property related to trem-cel, VCAR33 and VADC45 and assigned certain license agreements related to these product candidates to the buyers, including the exclusive license agreement with The Trustees of Columbia University in the City of New York and patent license agreement with the U.S. Department of Health and Human Services, as represented by National Cancer Institute of the National Institutes of Health.

Recent Events

On March 26, 2026, we entered into a securities purchase agreement (the "Purchase Agreement") with entities affiliated with TCGX pursuant to which we agreed to issue and sell, in a private placement, an aggregate of

5,338,078 shares (the "Shares") of common stock, at a price per Share of $14.05, for gross proceeds of approximately $75.0 million (the "2026 Private Placement"). The purchase agreement contains customary representations and warranties of the Company, on the one hand, and the investors, on the other hand, and customary conditions to closing. The closing of the 2026 Private Placement is expected to occur on March 30, 2026, subject to satisfaction of closing conditions.

In connection with the 2026 Private Placement, we also entered into a registration rights agreement, dated March 26, 2026 (the "Registration Rights Agreement"), with the investors in the 2026 Private Placement. Pursuant to the terms of the Registration Rights Agreement, we agreed to prepare and file with the Securities and Exchange Commission ("SEC") a registration statement on Form S-3 (the "Private Placement Registration Statement") to register for resale the Shares within 30 days of the closing date of the 2026 Private Placement and to use our reasonable best efforts to have the Private Placement Registration Statement declared effective at the earliest possible date, but no later than 60 days after the closing date of the 2026 Private Placement, subject to extension under the terms of the Registration Rights Agreement. The Registration Rights Agreement provides for liquidated damages if we fail to meet certain filing or effectiveness deadlines, subject to specified caps. The Registration Rights Agreement includes customary provisions regarding payment of fees and expenses and indemnification.

Sales and Marketing

Given our stage of development, we have not yet established a commercial organization or distribution capabilities. We plan to build focused capabilities in various regions worldwide (the United States, South America, Europe, Japan, the Middle East and North Africa) to commercialize our product candidate, if approved. We plan to focus our efforts in these regions because we believe the patient populations and medical specialists for the indications we are targeting are sufficiently concentrated to allow us to effectively promote our product candidate, if approved for commercial sale, with a targeted sales team.

Manufacturing

We plan to rely on third-party contract manufacturers for clinical manufacturing of required raw materials, manufacturing devices, active pharmaceutical ingredients and finished product for our research and clinical manufacturing. We do not have long-term agreements with any of these third parties. We also do not have any current contractual relationship for the manufacture of material for clinical trials beyond Phase 3 or commercial supplies. We intend to enter into agreements with third-party contract manufacturers and one or more backup manufacturers for future production. We continue to analyze the feasibility of building manufacturing capabilities for future development and commercial quantities of any products that we develop. Such products will need to be manufactured in facilities, and by processes, that comply with the requirements of the FDA and the regulatory authorities of other jurisdictions in which we are seeking approval.

Competition

The autoimmune field is a competitive landscape involving multiple fusion protein class members (atacicept, povetacicept), monoclonal antibodies, other biologics, chimeric antigen receptor (CAR) T cells and small molecules either already marketed or in development by many different companies including, but not limited to Alexion, Amgen, Argenx, Dianthus, Johnson & Johnson, UCB, and Vertex.

Reverse Stock Split

On September 18, 2025, the Company effected a 1-for-20 reverse stock split of its common stock. The par value and the number of authorized shares of common stock were not adjusted as a result of the reverse stock split. All share and per share amounts for all periods presented in this Annual Report, including the consolidated financial statements and the notes thereto, have been adjusted retroactively, where applicable, to reflect the effect of this reverse stock split.

License Agreements

Telitacicept License Agreement

In June 2025, we entered into a license agreement with RemeGen (the "Telitacicept License Agreement") granting us an exclusive (even as to RemeGen) license under RemeGen's patents and know-how to exploit, develop and commercialize telitacicept and related products in all territories other than Greater China, with the right to grant sublicenses. We also received a non-exclusive license to manufacture the licensed products worldwide solely for use in the licensed territory. In exchange, RemeGen received an exclusive, perpetual and irrevocable license under our intellectual property to exploit and manufacture the licensed products in Greater China, as well as a non-exclusive, fully paid-up license to manufacture the licensed products worldwide for use in Greater China. We are responsible for all development, regulatory and commercialization activities and costs in the licensed territory, including the conduct of clinical trials and regulatory submissions.

As consideration for the Telitacicept License Agreement, we paid RemeGen an upfront payment of $125 million, consisting of a cash payment of $45 million and the issuance of $80 million in equity to a subsidiary of RemeGen in the form of a warrant to purchase 16,000,000 shares of our common stock at an exercise price of $0.0001 per share. RemeGen is eligible to receive up to $330 million in regulatory milestone payments and up to $3.775 billion in sales milestone payments. In addition, RemeGen is entitled to receive tiered royalties on net sales of the licensed products in the licensed territory, ranging from high single digit to mid-teen percentages of net sales, subject to customary reductions. If we enter into a sublicense or divest rights to the licensed products prior to a specified development event and other than in connection with a change of control, RemeGen is entitled to receive a single digit percentage of certain net proceeds from such transaction.

The Telitacicept License Agreement may be terminated, in its entirety or on a region-by-region basis, by either party for material breach (subject to cure periods and dispute resolution) or insolvency of the other party, by us for convenience with advance notice, or by RemeGen if we challenge the validity of licensed patents. Upon termination, all rights and licenses in the terminated region will revert to RemeGen, with a wind-down period for us to cease activities.

Intellectual Property

The patent portfolio licensed from RemeGen includes a first patent family directed to the telitacicept composition of matter, with a granted patent in each of the United States, China, Europe, Japan, Korea, Russia, Brazil, and India. The patents granted in this family outside of China are expected to expire in 2028, absent any applicable patent term extensions.

Three additional families licensed from RemeGen are directed to formulations of telitacicept. A first family covering aqueous liquid formulations includes applications pending in the United States, Canada, Brazil, India, Singapore, Korea, and Hong Kong with granted patents in each of the United States, Australia, Russia, China, Japan, and Europe. Any patents that grant from applications claiming priority to this patent family would be expected to expire in 2040, absent any applicable patent term extensions.

A second family covering liquid preparations includes applications pending in the United States, Canada, Russia, Australia, Europe, India, Brazil, Japan, China, Indonesia, Mexico, Korea, Israel, and Singapore, with a granted patent in Taiwan. Any patents that grant from applications claiming priority to this patent family would be expected to expire in 2043, absent any applicable patent term extensions. A third family covering protected liquid formulations of telitacicept includes a pending PCT international application. Any patents that grant from applications claiming priority to this patent family would be expected to expire in 2044, absent any applicable patent term extensions.

Nine additional families licensed from RemeGen are directed to methods and uses of telitacicept for the treatment of specific conditions. A first family covering the treatment of systemic lupus erythematosus includes applications filed in the United States, Canada, Korea, and Singapore, with granted patents in Russia and Australia. Any patents that grant from applications claiming priority to this patent family would be expected to expire in 2039, absent any applicable patent term extensions.

A second family covering the treatment of IgA nephropathy includes applications filed in the United States, Russia, Australia, Canada, Brazil, Europe, Korea, Singapore, Hong Kong, and Taiwan, with a granted patent in Japan. A third family covering the treatment of Sjogren's syndrome includes applications filed in the United States, Australia, Canada, Brazil, Europe, Korea, Japan, Singapore, China, Hong Kong, and Taiwan, with a granted patent in Russia. Any patents that grant from applications claiming priority to these patent families would be expected to expire in 2042, absent any applicable patent term extensions.

A fourth family covering the treatment of myasthenia gravis includes applications filed in the United States, Canada, Australia, Russia, Europe, Brazil, Japan, China, Singapore, Korea, Indonesia, Mexico, Israel, and Hong Kong, with a granted patent in Taiwan. A fifth family covering the treatment of membranous nephropathy includes applications filed in the United States, Taiwan, China, Europe, and Japan. Any patents that grant from applications claiming priority to these patent families would be expected to expire in 2043, absent any applicable patent term extensions. A sixth family covering the treatment of ANCA-associated vasculitis includes applications filed in the United States, Europe, Japan, Taiwan, and China. Any patents that grant from applications claiming priority to these patent families would be expected to expire in 2044, absent any applicable patent term extensions.

A seventh family covering the treatment of antibody-mediated rejection includes a pending PCT international application and applications in Taiwan and China. Any patents that grant from applications claiming priority to these patent families would be expected to expire in 2044, absent any applicable patent term extensions. An eighth family covering the treatment of autoimmune encephalitis includes a pending PCT international application and an application in China. Any patents that grant from applications claiming priority to these patent families would be expected to expire in 2044, absent any applicable patent term extensions. A ninth family covering the treatment of antiphospholipid syndrome includes a pending PCT international application. Any patents that grant from applications claiming priority to these patent families would be expected to expire in 2045, absent any applicable patent term extensions.

Government Regulation

Government authorities in the United States at the federal, state and local level and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of drug and biological products, such as our investigational medicines and any future investigational medicines. Generally, before a new drug or biologic can be marketed, considerable data demonstrating its quality, safety and efficacy must be obtained, organized into a format specific for each regulatory authority, submitted for review and approved by the regulatory authority.

Regulatory Approval in the United States

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug and Cosmetic Act ("FDCA"), and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post- approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Biological products used for the prevention, treatment or cure of a disease or condition of a human being are subject to regulation under the FDCA, except that they are approved, or licensed, for marketing under provisions of the Public Health Service Act ("PHSA") via a BLA. The application process and requirements for approval of BLAs for reference biological products are similar to those for New Drug Applications for new chemical entities. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as clinical hold, FDA refusal to approve pending BLAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, and criminal prosecution.

Our investigational medicines and any future investigational medicines must be approved by the FDA pursuant to a BLA before they may be legally marketed in the United States. The process generally involves the following:

- completion of extensive preclinical laboratory and animal studies in accordance with applicable regulations, including studies conducted in accordance with good laboratory practices ("GLP") requirements;
- submission to the FDA of an Investigational New Drug Application ("IND"), which must become effective before human clinical trials may begin;
- approval of the protocol and related documents by an institutional review board ("IRB") or independent ethics committee at each clinical trial site before each clinical trial may be commenced;

- performance of adequate and well controlled human clinical trials in accordance with applicable IND regulations, GCP requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- preparation of and submission to the FDA of a BLA for marketing approval that includes sufficient evidence of establishing the safety, purity, and potency of the proposed biological product for its intended indication, including from results of nonclinical testing and clinical trials;
- payment of any user fees for FDA review of the BLA;
- a determination by the FDA within 60 days of its receipt of a BLA to accept the filing for review;
- satisfactory completion of one or more FDA pre-approval inspections of the manufacturing facility or facilities where the biologic, or components thereof, will be produced to assess compliance with current cGMP requirements to assure that the facilities, methods and controls are adequate to preserve the biologic's identity, strength, quality and purity;
- satisfactory completion of any potential FDA audits of the clinical trial sites that generated the data in support of the BLA to assure compliance with GCPs and integrity of the clinical data;
- potential FDA audit of the nonclinical study and clinical trial sites that generated the data in support of the BLA;
- FDA review and approval of the BLA, including consideration of the views of any FDA advisory committee; and
- compliance with any post-approval requirements, including a REMS, where applicable, and post-approval studies required by the FDA as a condition of approval.

The preclinical and clinical testing and approval process requires substantial time, effort and financial resources, and we cannot be certain that any approvals for our product candidates will be granted on a timely basis, or at all.

Preclinical studies

Before testing any biological product candidates in humans, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as in vitro and animal studies to assess the potential for adverse events and in some cases to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations for safety/toxicology studies.

Prior to beginning the first clinical trial with a product candidate in the United States, an IND must be submitted to the FDA and the FDA must allow the IND to proceed. An IND is an exemption from the FDCA that allows an unapproved product candidate to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA allowance that such investigational product may be administered to humans in connection with such trial. Such authorization must be secured prior to interstate shipment and administration. In support of a request for an IND, applicants must submit a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. An IND sponsor must also submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, to the FDA as part of an IND. Some long-term preclinical testing may continue after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical trials

The clinical stage of development involves the administration of the investigational product to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control. Clinical trials must be conducted: (i) in compliance with federal regulations; (ii) in

compliance with GCPs, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators and monitors; as well as (iii) under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated in the trial. Each protocol involving testing on U.S. patients and subsequent protocol amendments 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 trials are minimized and are reasonable in relation to 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. An IRB must operate in compliance with FDA regulations. An IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product candidate has been associated with unexpected serious harm to patients.

Some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee ("DSMB"). This group provides authorization as to whether or not a trial may move forward at designated check points based on access that only the group maintains to available data from the study.

There also are requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Information about certain clinical trials, including clinical trial results, must be submitted within specific timeframes for publication on the www.clinicaltrials.gov website. Information related to the product, patient population, phase of investigation, clinical trial sites and investigators and other aspects of the clinical trial is then made public as part of the registration. Disclosure of the results of these clinical trials can be delayed in certain circumstances for up to two years after the date of completion of the trial.

A sponsor who wishes to conduct a clinical trial outside of the United States 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 may submit data from the clinical trial to the FDA in support of a BLA. The FDA will accept a well-designed and well-conducted foreign clinical trial not conducted under an IND if the clinical 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 are generally conducted in three sequential phases, known as Phase 1, Phase 2 and Phase 3:

- Phase 1 clinical trials generally involve a small number of healthy volunteers or disease-affected patients who are initially exposed to a single dose and then multiple doses of the product candidate. The primary purpose of these clinical trials is to assess the metabolism, pharmacokinetics, pharmacologic action, side effect tolerability, safety of the product candidate, and, if possible, early evidence of effectiveness.
- Phase 2 clinical trials generally involve studies in disease-affected patients to evaluate proof of concept and/or determine the dosing regimen(s) for subsequent investigations. At the same time, safety and further pharmacokinetic and pharmacodynamic information is collected, possible adverse effects and safety risks are identified, and a preliminary evaluation of efficacy is conducted.
- Phase 3 clinical trials generally involve a large number of patients at multiple sites and are designed to provide the data necessary to demonstrate the effectiveness of the product for its intended use, its safety in use and to establish the overall benefit/risk relationship of the product and provide an adequate basis for product labeling. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the biologic.

These Phases may overlap or be combined. For example, a Phase 1/2 clinical trial may contain both a dose-escalation stage and a dose-expansion stage, the latter of which may confirm tolerability at the recommended dose for expansion in future clinical trials.

A single Phase 3 or Phase 2 trial with other confirmatory evidence may be sufficient in rare instances to provide substantial evidence of effectiveness (generally subject to the requirement of additional post-approval studies).

In some cases, FDA may require, or firms may voluntarily pursue, post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information.

Phase 1, Phase 2, Phase 3 and other types of clinical trials may not be completed successfully within any specified period, if at all. The FDA, the IRB, or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including non-compliance with regulatory requirements or a finding that the patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug or biologic has been associated with unexpected serious harm to patients.

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

FDA review processes

Following completion of the clinical trials, the results of preclinical studies and clinical trials are submitted to the FDA as part of a BLA, along with proposed labeling, chemistry and manufacturing information to ensure product quality and other relevant data. 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 a BLA must be obtained before a biologic may be marketed in the United States.

The cost of preparing and submitting a BLA is substantial. Under the Prescription Drug User Fee Act ("PDUFA"), each BLA 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 BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication. The applicant under an approved BLA is also subject to an annual program fee.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the FDA accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review to determine if it is substantially complete before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe, pure and potent, for its intended use, and whether the product is being manufactured in accordance with cGMP to ensure its continued safety, purity and potency.

Under the goals and policies agreed to by the FDA under PDUFA, the FDA has 10 months, from the filing date, in which to complete its initial review of a BLA and respond to the applicant, and six months from the filing date of a BLA designated for priority review. The review process for both standard and priority review may be extended by the FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission. The FDA does not always meet its PDUFA goal

dates for standard and priority BLAs, and the review process can be extended by FDA requests for additional information or clarification.

Before approving a BLA, the FDA will conduct a pre-approval inspection of the manufacturing facilities for the new product to determine whether they comply with cGMP requirements and to ensure they can supply the market demand once approved. The FDA will not approve the product 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.

The FDA also may audit data from clinical trials to ensure compliance with GCP requirements and the integrity of the data supporting safety and efficacy. Additionally, the FDA may refer applications for novel products or products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions, if any. The FDA is not bound by recommendations of an advisory committee, but it generally follows such recommendations when making decisions on approval. The FDA likely will reanalyze the clinical trial data, which could result in extensive discussions between the FDA and the applicant during the review process.

After the FDA evaluates a BLA, it will issue either an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the biologic with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter generally outlines the deficiencies in the BLA and may require additional clinical data, additional pivotal clinical trial(s) and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing in order for FDA to reconsider the application. If a Complete Response Letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application or request an opportunity for a hearing. The FDA has committed to reviewing such resubmissions in two or six months, depending on the type of information included. Even if such data and information are submitted, the FDA may decide that the BLA does not satisfy the criteria for approval.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, including to subpopulations of patients, which could restrict the commercial value of the product. Furthermore, as a condition of BLA approval, the FDA may require a REMS to help ensure that the benefits of the biologic outweigh the potential risks to patients. A REMS can include medication guides, communication plans for healthcare professionals and elements to assure a product's safe use ("ETASU"). An ETASU can include, but is not limited to, special training or certification for prescribing or dispensing the product, dispensing the product only under certain circumstances, special monitoring and the use of patient-specific registries. The requirement for a REMS can materially affect the potential market and profitability of the product. Moreover, the FDA may require substantial post-approval testing and surveillance to monitor the product's safety or efficacy.

Orphan drug designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States but for which there is no reasonable expectation that the cost of developing and making the product for this type of disease or condition will be recovered from sales of the product in the United States.

Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation on its own does not convey any advantage in or shorten the duration of the regulatory review and approval process.

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 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 from the date of such approval, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity by means of greater effectiveness, greater safety, or providing a major contribution

to patient care, or in instances of drug supply issues. A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Orphan drug exclusivity may be lost if the FDA later determines that the request for designation was materially defective. Further, competitors may receive approval of either a different product for the same indication or the same product for a different indication. In the latter case, because healthcare professionals are free to prescribe products for off-label uses, the competitor's product could be used for the orphan indication despite another product's orphan exclusivity.

Expedited development and review programs

The FDA is authorized to designate certain products for expedited review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition.

Fast track designation may be granted for products that are intended to treat a serious or life-threatening disease or condition for which there is no effective treatment and preclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast track designation applies to both the product and the specific indication for which it is being studied. The sponsor of a new biologic candidate can request the FDA to designate the candidate for a specific indication for fast track status concurrent with, or after, the submission of the IND for the candidate. The FDA must determine if the biologic candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request. For fast track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a fast track product's BLA before the application is complete. This "rolling review" is available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. Any product submitted to the FDA for marketing, including under a fast track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval.

Breakthrough therapy designation may be granted for products that are intended, alone or in combination with one or more other products, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints. Under the breakthrough therapy program, the sponsor of a new biologic candidate may request that the FDA designate the candidate for a specific indication as a breakthrough therapy concurrent with, or after, the submission of the IND for the biologic candidate. The FDA must determine if the biological product qualifies for breakthrough therapy designation within 60 days of receipt of the sponsor's request. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process, providing timely advice to the product sponsor regarding development and approval, involving more senior staff in the review process, assigning a cross-disciplinary project lead for the review team and taking other steps to design the clinical trials in an efficient manner.

Priority review may be granted for products that are intended to treat a serious or life-threatening condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA will attempt to direct additional resources to the evaluation of an application designated for priority review in an effort to facilitate the review. Under priority review, the FDA's goal is to review an application in six months once it is filed, compared to ten months for a standard review. Priority review designation does not change the standard for approval or the quality of evidence necessary to support approval.

Accelerated approval may be granted for products that are intended to treat a serious or life-threatening condition and that generally provide a meaningful therapeutic advantage to patients over existing treatments. A product eligible for accelerated approval may be approved on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that 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. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions or survives. The accelerated approval pathway is most often used in settings in which the course of a disease is long, and an extended period of time is required to measure the intended clinical benefit of a product, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Use of the accelerated approval pathway entails submission of a BLA with the surrogate or intermediate clinical endpoint data while continuing to conduct the trial(s) to completion and is

contingent on a sponsor's agreement to complete and/or conduct additional post-approval confirmatory studies to verify and describe the product's clinical benefit. These confirmatory trials must be completed with due diligence and, in some cases, the FDA may require that the trial be designed, initiated and/or fully enrolled prior to approval. Failure to conduct required post-approval studies, or to confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the product from the market on an expedited basis. All promotional materials for product candidates approved under accelerated regulations are subject to prior review by the FDA.

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 standards for approval, but may expedite the development or approval process.

Additional controls for biologics

To help reduce the increased risk of the introduction of adventitious agents, the PHSA emphasizes the importance of manufacturing controls for products whose attributes cannot be precisely defined. The PHSA also provides authority to the FDA to immediately suspend licenses in situations where there exists a danger to public health, to prepare or procure products in the event of shortages and critical public health needs, and to authorize the creation and enforcement of regulations to prevent the introduction or spread of communicable diseases in the United States and between states.

After a BLA is approved, the product may also be subject to official lot release as a condition of approval. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA may also perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency and effectiveness of biological products. As with drugs, after approval of biologics, manufacturers must address any safety issues that arise, are subject to recalls or a halt in manufacturing, and are subject to periodic inspection after approval.

Post-approval requirements

Once a BLA is approved, a product may be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of biologics, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the Internet. Biologics may be marketed only for the approved indications and in a manner consistent with the provisions of the approved labeling. Although physicians may prescribe products for off-label uses as the FDA and other regulatory authorities do not regulate a physician's choice of drug treatment made in the physician's independent medical judgment, they do restrict promotional communications from companies or their sales force with respect to off-label uses of products for which marketing clearance has not been issued. Companies may only share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling.

Adverse event reporting and submission of periodic safety summary reports is required following FDA approval of a BLA. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, and surveillance to monitor the effects of an approved product, or the FDA may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control, biological product manufacture, packaging and labeling procedures must continue to conform to cGMPs after approval. Biologic manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects a biologic product's manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with required regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical trials to assess new safety risks or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, suspension of the approval, complete withdrawal of the product from the market or product recalls;
- fines, warning or other enforcement-related letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending BLAs or supplements to approved BLAs, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

U.S. marketing exclusivity

The Biologics Price Competition and Innovation Act of 2009 ("BPCIA") created an abbreviated approval pathway for biological products shown to be biosimilar to, or interchangeable with, an FDA-licensed reference biological product. Biosimilarity, which requires that the biological product be highly similar to the reference product notwithstanding minor differences in clinically inactive components and that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity and potency, can be shown through analytical studies, animal studies and a clinical trial or trials. Interchangeability requires that a biological product be biosimilar to the reference product and that the product can be expected to produce the same clinical results as the reference product in any given patient and, for products administered multiple times to an individual, that the product and the reference product may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biological product without such alternation or switch.

A reference biological product is granted 12 years of data exclusivity from the time of first licensure of the product and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product. "First licensure" typically means the initial date the particular product at issue was licensed in the United States. Date of first licensure does not include the date of licensure of (and a new period of exclusivity is not available for) a biological product if the licensure is for a supplement for the biological product or for a subsequent application by the same sponsor or manufacturer of the biological product (or licensor, predecessor in interest or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength, or for a modification to the structure of the biological product that does not result in a change in safety, purity or potency.

Regulatory approval in the European Union

The EMA is a decentralized scientific agency of the European Union ("EU"). It coordinates the evaluation and monitoring of centrally authorized medicinal products. It is responsible for the scientific evaluation of applications for EU marketing authorizations, as well as the development of technical guidance and the provision of scientific advice to sponsors. The EMA decentralizes its scientific assessment of medicines by working through a network of about 4,500 experts throughout the European Union, nominated by the Member States. The EMA draws on resources of over 40 national competent authorities of European Union Member States.

The process regarding approval of medicinal products in the European Union follows roughly the same lines as in the United States and likewise generally involves satisfactorily completing each of the following:

- preclinical laboratory tests, animal studies and formulation studies all performed in accordance with the applicable EU Good Laboratory Practice regulations;

- submission of a single clinical trial application ("CTA") through the Clinical Trials Information System ("CTIS") to the relevant national authorities of EU Member States in which a clinical trial is planned to be conducted, which must be approved by such national authorities and the subject of a positive opinion from at least one independent ethics committee before the trial may begin in each country where the clinical trial is planned;
- performance of adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication;
- submission to the relevant competent authorities of a marketing authorization application ("MAA") which includes the data supporting safety and efficacy as well as detailed information on the manufacture and composition of the product in clinical development and proposed labelling;
- satisfactory completion of an inspection by the relevant competent national authorities of EU Member States of the manufacturing facility or facilities, including those of third parties, at which the product is produced to assess compliance with strictly enforced cGMP;
- potential audits of the non-clinical and clinical trial sites that generated the data in support of the MAA; and
- review and approval by the relevant competent authority of the MAA before any commercial marketing, sale or shipment of the product.

Preclinical studies

Preclinical tests include laboratory evaluations of product chemistry, formulation and stability, as well as studies to evaluate toxicity in animal studies, in order to assess the quality and potential safety and efficacy of the product. The conduct of the preclinical tests and formulation of the compounds for testing must comply with the relevant international, EU and national legislation, regulations and guidelines. The results of the preclinical tests, together with relevant manufacturing information and analytical data, are submitted as part of the CTA.

Clinical trials

Similarly to the United States, the various phases of non-clinical and clinical research in the EU are subject to significant regulatory controls.

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

The CTR is intended to harmonize and streamline clinical trial authorizations, simplify adverse-event reporting procedures, improve the supervision of clinical trials and increase 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 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.

The CTR foresaw a three-year transition period that ended on January 31, 2025. Since this date, all new or ongoing trials are subject to the provisions of the CTR.

Review and approval

In the EU, medicinal products can only be commercialized after a related marketing authorization ("MA") has been granted. To obtain an MA for a product in the EU, an applicant must submit a Marketing Authorization Application ("MAA"), either under a centralized procedure administered by the EMA, or one of the procedures

administered by the competent authorities of EU Member States (decentralized procedure, national procedure or mutual recognition procedure). An MA may be granted only to an applicant established in the EU.

The centralized procedure provides for the grant of a single MA by the European Commission that is valid throughout the European Economic Area ("EEA"), which is comprised of the 27 EU Member States plus Iceland, Liechtenstein and Norway. 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 ("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 authorization procedure, the EMA's Committee for Medicinal Products for Human Use ("CHMP") conducts the initial assessment of a product. The CHMP is composed of experts nominated by each member state's national drug authority, with one of them appointed to act as Rapporteur for the co-ordination of the evaluation with the possible assistance of a further member of the CHMP acting as a Co-Rapporteur. After approval, the Rapporteur(s) continue to monitor the product throughout its life cycle. The CHMP is required to issue an opinion within 210 days of receipt of a valid application, though the clock is stopped if it is necessary to ask the applicant for clarification or further supporting data. Clock stops may extend the timeframe of evaluation of a marketing authorization application considerably beyond 210 days. The process is complex and involves extensive consultation with the regulatory authorities of Member States and a number of experts. Once the procedure is completed, a European Public Assessment Report is produced. If the CHMP concludes that the quality, safety and efficacy of the medicinal product is sufficiently proven, it adopts a positive opinion. The CHMP's opinion is sent to the European Commission, which uses the opinion as the basis for its decision whether or not to grant a marketing authorization. The European Commission's decision is issued within 67 days of receipt of the CHMP's recommendation. If the opinion is negative, information is given as to the grounds on which this conclusion was reached.

After a drug has been authorized and launched, it is a condition of maintaining the marketing authorization that all aspects relating to its quality, safety and efficacy must be kept under review. Sanctions may be imposed for failure to adhere to the conditions of the marketing authorization. In extreme cases, the authorization may be revoked, resulting in withdrawal of the product from sale.

Validity of marketing authorizations

A marketing authorization has, in principle, an initial validity of five years. The market authorization 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 EU Member State in which the original MA was granted. To support the application, the market authorization holder must provide the EMA or the competent authority with a consolidated version of the Common Technical Document providing up-to-date data concerning the quality, safety and efficacy of the product, including all variations introduced since the marketing authorization was granted, at least nine months before the marketing authorization ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide, on justified grounds relating to pharmacovigilance, to proceed with one additional five-year renewal period for the marketing authorization. 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 procedure marketing authorization) or on the market of the authorizing EU Member State within three years after authorization ceases to be valid (the so-called sunset clause).

Manufacturing regulation in the EU

In addition to a marketing authorization, various other requirements apply to the manufacturing and placing on the EU market of medicinal products. The manufacturing of medicinal products in the EU requires a manufacturing authorization and import of medicinal products into the EU requires a manufacturing authorization allowing for import. The manufacturing authorization holder must comply with various requirements set out in the applicable EU laws, regulations and guidance, including EU cGMP standards. Similarly, the distribution of medicinal products within the EU is subject to compliance with the applicable EU laws, regulations and guidelines, including the requirement to hold appropriate authorizations for distribution granted by the competent authorities of

EU Member States. Marketing authorization holders and/or manufacturing and import authorization, or marketing authorization holders and/or distribution authorization holders may be subject to civil, criminal or administrative sanctions, including suspension of manufacturing authorization, in case of non-compliance with the EU or EU Member States' requirements applicable to the manufacturing of medicinal products.

Data and market exclusivity

The EU provides opportunities for data and market exclusivity related to marketing authorizations. Upon receiving a marketing authorization, innovative medicinal products are generally entitled to receive eight years of data exclusivity and 10 years of market exclusivity. Data exclusivity, if granted, prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic application or biosimilar application for eight years from the date of authorization of the innovative product, after which a generic or biosimilar MAA can 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 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 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. However, there is no guarantee that a product will be considered by the EU's regulatory authorities to be a new chemical/biological entity, and products may not qualify for data exclusivity.

In the EU, 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.

Orphan drug designation

In 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 medicinal product by the European Commission if its sponsor can establish that (i) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (ii) either (a) such condition affects not more than five in 10,000 persons in the European Union when the application is made, or, (b) the product without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify the necessary investment in its development; and (iii) there exists no satisfactory authorized method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Union or even, if such method exists, the product will be of significant benefit to those affected by that condition.

Regulation (EC) No. 847/2000 sets out further provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product. An application for designation as an orphan product can be made any time prior to the submission of an MAA. A marketing authorization for an orphan medicinal product may only include indications designated as orphan. For non-orphan indications treated with the same active pharmaceutical ingredient, a separate marketing authorization has to be sought.

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 a grant of marketing authorization, orphan medicinal products are entitled to a 10-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 PIP. 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 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 designation, 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, an MA may be granted to a similar medicinal product with the same orphan indication during the 10-year period if (i) 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.

Post-authorization requirements

Where an MA is granted in relation to a medicinal product in the EU, 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 EU Member States. 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 (PSURs).

All new MAAs must include a risk management plan (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 EU, the advertising and promotion of medicinal products are subject to both EU and EU Member States' laws governing promotion of medicinal products, interactions with physicians and other healthcare professionals, misleading and comparative advertising and unfair commercial practices. General requirements for advertising and promotion of medicinal products, such as direct-to-consumer advertising of prescription medicinal products are established in EU law. However, the details are governed by regulations in individual EU Member States 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 (SmPC), which may require approval by the competent national authorities in connection with an MA. The SmPC is the document that provides information to physicians and other health care professionals 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 EU.

European data collection and processing

The collection, receipt, storage, generation, transfer, access, protection, securing, disposal, transmittal, sharing, use, disclosure and other processing (commonly referred to as processing) of health-related and other personal data about clinical trials participants and other individuals in Europe is governed by the European Union's General Data Protection Regulation ("EU GDPR"). The EU GDPR requires companies to, among other things, give detailed disclosures about how they are processing personal data; ensure any consents relied on to process personal data (including special categories of personal data, such as health information) meet the strict EU GDPR requirements; contractually impose data protection measures on vendors entrusted with personal data; maintain adequate data security measures; notify regulators and affected individuals of certain data breaches; meet extensive privacy governance and documentation requirements; honor individuals' data protection rights, including their rights to access, correct and delete their personal data; and refrain from transferring personal data from Europe to most other countries unless specific safeguards can be implemented. Companies that violate the EU GDPR can face private litigation, prohibitions on data processing and heavy fines. Complying with the EU GDPR may be costly and require us to limit our activities in Europe. If our efforts to comply are not successful, we may face litigation, reputational harm, significant penalties and other liabilities.

Marketing

Much like the Anti-Kickback Statute prohibition in the United States, as described below, the provision of benefits or advantages to physicians and other health care professionals to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU.

Interactions between pharmaceutical companies and health care professionals are governed by strict laws, such as national anti-bribery laws of European countries, national sunshine rules, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment. Infringement of related laws could result in substantial fines and imprisonment.

Payments made to physicians and other health care professionals in certain European Union Member States must be publicly disclosed. Moreover, agreements with health care professionals may require prior notification or approval by the health care professional's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

International regulation

In addition to regulations in the United States and Europe, a variety of foreign regulations govern clinical trials, commercial sales and distribution of product candidates. The approval process varies from country to country and the time to approval may be longer or shorter than that required for FDA or European Commission approval.

Other healthcare laws and regulations and legislative reform

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our operations, including any arrangements with healthcare providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws that may affect the business or financial arrangements and relationships through which we conduct research and would market, sell and distribute our products. The healthcare laws that may affect our ability to operate include, but are not limited to:

- The federal Anti-Kickback Statute, which prohibits any person or entity from, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of an item or service reimbursable, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. The term "remuneration" has been broadly interpreted to include anything of value. The federal Anti-Kickback Statute has also been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other hand. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, but the exceptions and safe harbors are drawn narrowly and require strict compliance in order to offer protection. Additionally, the intent standard under the federal Anti-Kickback Statute was amended by the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, Affordable Care Act), to a stricter standard such that a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Further, the Affordable Care Act codified case law that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act.
- Federal civil and criminal false claims laws, such as the False Claims Act, which can be enforced by private citizens through civil qui tam actions, and civil monetary penalty laws prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, false, fictitious or fraudulent claims for payment of federal funds, and knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to avoid, decrease or conceal an obligation to pay money to the federal government. For example, pharmaceutical companies have been prosecuted under the False Claims Act in connection with their alleged off-label promotion of drugs, purportedly concealing price concessions in the pricing information submitted to the government for government price reporting purposes, and allegedly providing free product to customers with the expectation that the customers would bill federal healthcare programs for the product. In addition, a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. As a result of a modification

made by the Fraud Enforcement and Recovery Act of 2009, a claim includes “any request or demand” for money or property presented to the U.S. government. In addition, manufacturers can be held liable under the False Claims Act even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims.

- The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), among other things, imposes criminal liability for executing or attempting to execute a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and creates federal criminal laws that prohibit knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement or representation, or making or using any false writing or document knowing the same to contain any materially false, fictitious or fraudulent statement or entry in connection with the delivery of or payment for healthcare benefits, items or services.
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH), and their implementing regulations, which impose privacy, security and data breach reporting obligations with respect to individually identifiable health information upon entities subject to the law, such as health plans, healthcare clearinghouses and certain healthcare providers, known as covered entities, and their respective business associates and subcontractors that perform services for them that involve individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in U.S. federal courts to enforce HIPAA laws and seek attorneys’ fees and costs associated with pursuing federal civil actions.
- Federal and state consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.
- The federal transparency requirements under the Physician Payments Sunshine Act, created under the Affordable Care Act, which requires, among other things, certain manufacturers of drugs, devices, biologics and medical supplies reimbursed under Medicare, Medicaid, or the Children’s Health Insurance Program to report annually to the Centers for Medicare & Medicaid Services (CMS) information related to payments and other transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other health care professionals (such as physician assistants and nurse practitioners) and teaching hospitals, as well as information regarding ownership and investment interests held by physicians and their immediate family members.
- State and foreign laws that are analogous to each of the above federal laws, such as anti-kickback and false claims laws, that may impose similar or more prohibitive restrictions, and may apply to items or services reimbursed by non-governmental third-party payors, including private insurers.
- State and foreign laws that require pharmaceutical companies to implement compliance programs, comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the government, or to track and report gifts, compensation and other remuneration provided to physicians and other healthcare professionals; state and foreign laws that require the reporting of marketing expenditures or drug pricing, including information pertaining to and justifying price increases; state and local laws that require certain regulatory licenses to manufacture or distribute our products commercially and/or the registration of pharmaceutical sales representatives; state and foreign laws that prohibit various marketing-related activities, such as the provision of certain kinds of gifts or meals; state and foreign laws that require the posting of information relating to clinical trials and their outcomes; and other federal, state and foreign laws that govern the privacy and security of health information or personal data in certain circumstances, including state health information privacy and data breach notification laws which govern the processing of health-related and other personal data, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus requiring additional compliance efforts.

If our operations are found to be in violation of any of these laws or any other current or future healthcare laws that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages,

fines, disgorgement, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, or comparable foreign programs, contractual damages, reputational harm, diminished profits and future earnings, 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, and the curtailment or restructuring of our operations, any of which could substantially disrupt our operations. Although effective compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses and could divert our management's attention from the operation of our business, even if our defense is successful. In addition, if any of the physicians or other healthcare professionals or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Legislative reform

We operate in a highly regulated industry, and new laws, regulations and judicial decisions, or new interpretations of existing laws, regulations and decisions, related to healthcare availability, the method of delivery and payment for healthcare products and services could negatively affect our business, financial condition and prospects. There is significant interest in promoting healthcare reforms, and it is likely that federal and state legislatures within the United States and the governments of other countries will continue to consider changes to existing healthcare legislation.

For example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. The Affordable Care Act, among other things, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly affected the pharmaceutical industry.

There have been executive, judicial and congressional challenges and amendments to certain aspects of the Affordable Care Act. For example, on July 4, 2025, the One Big Beautiful Bill Act (the "OBBBA") was signed into law, which narrowed access to ACA marketplace exchange enrollment and declined to extend the ACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired ACA subsidies.

In addition, there have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs. These changes include aggregate reductions to Medicare payments to providers of 2% per fiscal year, which began in 2013 and will remain in effect until 2032 unless additional Congressional action is taken.

The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at the U.S. Department of Health and Human Services ("HHS"), the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with several pharmaceutical companies that require the drug manufacturers to offer, through a direct to consumer platform ("TrumpRx") U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the Make America Healthy Again ("MAHA") Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager ("PBM") payment methodologies,

among other things. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In June 2024, the U.S. Supreme Court's Loper Bright decision greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

Environmental, health and safety laws and regulations

We and our third-party contractors are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the use, generation, manufacture, distribution, storage, handling, treatment, remediation and disposal of hazardous materials and wastes. Hazardous chemicals, including flammable and biological materials, are involved in certain aspects of our business, and we cannot eliminate the risk of injury or contamination from the use, generation, manufacture, distribution, storage, handling, treatment or disposal of hazardous materials and wastes. In particular, our product candidates use PBDs, which are highly potent cytotoxins that require special handling by our and our contractors' staff. In the event of contamination or injury, or failure to comply with environmental, health and safety laws and regulations, we could be held liable for any resulting damages, fines and penalties associated with such liability could exceed our assets and resources. Environmental, health and safety laws and regulations are becoming increasingly more stringent. We may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations.

Pharmaceutical coverage, pricing and reimbursement

The availability and extent of coverage and adequate reimbursement by governmental and private third-party payors are essential for most patients to be able to afford expensive medical treatments. In both domestic and foreign markets, sales of our product candidates will depend substantially on the extent to which the costs of our product candidates will be covered by third- party payors, such as government health programs, commercial insurance and managed healthcare organizations. These third-party payors decide which products will be covered and establish reimbursement levels for those products.

Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

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

Obtaining coverage approval and reimbursement for a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide supporting scientific, clinical and cost-effectiveness data for the use of our products to the payor. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement at a satisfactory level. If coverage and adequate reimbursement of our future products, if any, are unavailable or limited in scope or amount, such as may result where alternative or generic treatments are available, we may be unable to achieve or sustain profitability. Adverse coverage and reimbursement limitations may hinder our ability to recoup our investment in our product candidates, even if such product candidates obtain regulatory approval.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. There is no uniform policy for coverage and reimbursement in the United States and, as a result, coverage and reimbursement can differ significantly from payor to payor. In the United States, the principal decisions about reimbursement for new medicines are typically made by the CMS, which decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors often, but not always, follow the CMS's decisions regarding coverage and reimbursement. It is difficult to predict what third-party payors will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Further, one payor's determination to provide coverage

and adequate reimbursement for a product does not assure that other payors will also provide coverage and adequate reimbursement for that product. We may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our product candidates. There can be no assurance that our product candidates will be considered medically necessary or cost-effective. Therefore, it is possible that any of our product candidates, even if approved, may not be covered by third-party payors or the reimbursement limit may be so restrictive that we cannot commercialize the product candidates profitably.

Further, the increased emphasis on cost containment in the United States will put additional pressure on product pricing, reimbursement and usage. For example, the U.S. Department of Health and Human Services, or HHS, imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. HHS has also been empowered to negotiate the price of certain single-source biologics that have been on the market for at least eleven (11) years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis.

Reimbursement authorities in Europe may be more restrictive than payors in the United States. In Europe, pricing and reimbursement schemes vary widely from country to country. For example, some countries provide that products may be marketed only after an agreement on reimbursement price has been reached. Such pricing negotiations with governmental authorities can take considerable time after receipt of marketing approval for a product. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. In addition, the European Union provides options for its Member States to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union Member States may approve a specific price for a product, may adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for product but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. In addition, some EU Member States may require the completion of additional studies that compare the cost-effectiveness of a particular medicinal product candidate to currently available therapies. This Health Technology Assessment (HTA) process is conducted to assess the public health impact, therapeutic impact, and the economic and societal impact of use of a given medicinal product in the national healthcare systems of individual countries. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. In December 2021, Regulation No. 2021/2282 on Health Technology Assessment, or HTA Regulation, was adopted. The HTA Regulation 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 HTA Regulation has applied from January 12, 2025 although it will enter into force iteratively and initially apply to new active substances to treat cancer and to all advanced therapy medicinal products ("ATMPs"), it will then be expanded to orphan medicinal products in January 2028, and to all centrally authorized medicinal products as of 2030. Selected high-risk medical devices will also be assessed under the HTA Regulation as of 2026.

Reference pricing used by various European Union Member States and parallel distribution, or arbitrage between low-priced and high-priced Member States, can further reduce prices. Furthermore, many Member States in the European Union have increased the amount of discounts required on pharmaceutical products, and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many Member States in the European Union. The downward pressure on healthcare costs in general, and prescription products in particular, has become increasingly intense. As a result, there are increasingly higher barriers to entry for new products. There can be no assurance that any country that has reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries. Accordingly, the reimbursement for any products in Europe may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Furthermore, the containment of healthcare costs has become a priority of foreign and domestic governments as well as private third-party payors. The prices of drugs have been a focus in this effort. Governments and private third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for

particular medications, which could affect our ability to sell our product candidates profitably. We also expect to experience pricing pressures due to the trend towards managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. These and other cost-control initiatives could cause us to decrease the price we might establish for products, which could result in lower-than-anticipated product revenues. In addition, the publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if coverage and adequate reimbursement of our products is unavailable or limited in scope or amount, our revenues and the potential profitability of our product candidates in those countries would be negatively affected.

Employees and Human Capital Resources

Our human capital is integral to helping us achieve our mission of reinventing the treatment of autoimmune disease by tackling it at its root. We have built a culture of high performance based on our core values:

- *Ambition:* Bold, urgent, and relentless in pursuit of our mission to improve patients' lives
- *Excellence:* Driven by results, committed to quality, and grounded in scientific rigor
- *Integrity:* Honest, transparent, and guided by strong ethical principles in every decision and action
- *Ownership:* Accountable, proactive, and disciplined stewards of resources
- *Unity:* Collaborative, inclusive, and united as one team

Our human capital objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and new talent. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards.

As of March 3, 2026, we had 76 full-time employees, 19 of whom held an M.D. or Ph.D. degree and 30 of whom are engaged in research and development activities. None of our employees are represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

Corporate Information

We were incorporated under the laws of the State of Delaware on December 30, 2015. Our principal executive offices are located at 500 Boylston Street, Suite 1350, Boston, Massachusetts 02116 and our telephone number is 617-655-6580.

Available Information

We maintain an internet website at www.vorbio.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 Securities Exchange Act of 1934, as amended (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 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. We also make available, free of charge on our website, the reports filed with the SEC by our executive officers, directors and 10% stockholders pursuant to Section 16 under the Exchange Act as soon as reasonably practicable after copies of those filings are provided to us by those persons. 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, particularly the information in the section entitled "Investors," 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 a part of this Annual Report. Our website address is included in this Annual Report as an inactive technical reference only.

Item 1A. Risk Factors.

The following risk factors and other information included in this Annual Report on Form 10-K ("Annual Report"), including our financial statements and related notes thereto, should be carefully considered. The risks and uncertainties described below are not the only risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see the discussion regarding some of the forward-looking statements that are qualified by these risk factors contained elsewhere in this Annual Report. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant net losses since inception. We expect to incur net losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have not generated any revenue and have incurred significant operating losses. For the year ended December 31, 2025 and 2024 our net loss was $696.0 million and $116.9 million, respectively. As of December 31, 2025, we had an accumulated deficit of $1,153.0 million. We have financed our operations primarily through the sale of our capital stock, including the sale of warrants to purchase our common stock. We have devoted all of our efforts to organizing and staffing our company, business and scientific planning, raising capital, acquiring and developing technology, identifying potential product candidates, undertaking studies of potential product candidates and evaluating a clinical path for our pipeline programs. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- advance and complete clinical trials of telitacicept in gMG and SjD;
- initiate clinical development of telitacicept in additional indications;
- initiate additional research programs and development of other potential product candidates;
- initiate preclinical testing and clinical trials for any other product candidates we identify and develop;
- maintain, expand, enforce, defend and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- hire additional research and development and clinical personnel;
- hire commercial personnel and advance market access and reimbursement strategies;
- add operational, financial and management information systems and personnel, including personnel to support our product development;
- acquire or in-license product candidates, intellectual property and technologies;
- develop or in-license manufacturing and distribution technologies;
- rely on collaborators or other third parties to manufacture current good manufacturing practices ("cGMP") material for clinical trials or potential commercial sales;
- establish a commercialization infrastructure and develop internal and external manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;
- should we decide to do so and receive approval for any of our product candidates, build and maintain, or purchase and validate, commercial-scale manufacturing facilities designed to comply with cGMP requirements; and
- operate as a public company.

We have not completed clinical development of any product candidate and expect that it will be years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must develop and, either directly or through collaborators, eventually commercialize a product or products with significant market potential. This will require us to be successful in a range of challenging activities, including identifying product candidates, completing preclinical testing and clinical trials of product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in these activities and, even if we do, may never generate revenues that are significant or large enough to achieve profitability. Our product candidate and research programs are currently in clinical development. Because of the numerous risks and uncertainties associated with developing product candidates, we are unable to predict the extent of any future losses or when we will become profitable, if at all. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investments in us.

We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce or eliminate our research and product development programs or future commercialization efforts.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the clinical development of telitacicept for the treatment of gMG and SjD and initiate clinical development of telitacicept in additional indications, and otherwise continue to advance our research programs in support of our pipeline. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a collaborator. Further, we expect to continue to incur significant additional costs associated with operating as a public company this year and in future years. Accordingly, we will need to obtain substantial additional funding in order to maintain our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and product development programs or future commercialization efforts.

As of December 31, 2025, our cash, cash equivalents and marketable securities were $455.2 million. We expect that our existing cash, cash equivalents and marketable securities as of December 31, 2025, together with the expected proceeds from our 2026 Private Placement, will enable us to fund our operating expenses and capital expenditure requirements into early 2029. However, our operating plan may change as a result of factors currently unknown to us, and we may need to seek funding sooner than planned. Our future capital requirements will depend on many factors, including:

- the progress, results and costs of clinical trials for telitacicept;
- the costs of researching, developing, and initiating clinical trials of telitacicept in additional indications;
- the scope, progress, results, costs of discovery, acquisition or in-licensing, preclinical development, formulation, development and clinical trials for other product candidates;
- the costs of acquiring and expanding facilities to accommodate corporate, laboratory, and manufacturing needs;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights and defending intellectual property-related claims in the United States and internationally;
- the costs, timing and outcome of regulatory review of any product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, distribution, coverage and reimbursement for any product candidates for which we receive regulatory approval;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the success of our collaborations, including ones we may establish, and of our license agreements;

- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration agreements we enter;
- the extent to which we acquire or in-license product candidates, intellectual property and technologies;
- the extent to which we develop or in-license manufacturing and distribution technologies; and
- the costs of operating as a public company.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully develop product candidates and those are approved, we may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize product candidates. We cannot be certain that additional funding will be available on acceptable terms, or at all. We have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of product candidates or other research and development initiatives. Our license agreements and any future collaboration agreements may also be terminated if we are unable to meet the payment or other obligations under the agreements. We could be required to seek collaborators for product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, government or private party grants, debt financings, collaborations, strategic alliances and licensing arrangements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, including through the use of our at-the-market facility, our stockholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect rights of our common stockholders. 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, declaring dividends and possibly other restrictions.

For example, we have raised substantial amounts of capital through the issuance and sale of 11,500,000 shares of common stock in the November 2025 Offering (as defined below), the issuance and sale of 13,876,032 shares of common stock in the December 2025 Private Placement (as defined below), the 2026 Private Placement and sales made pursuant to our at-the-market facility. In addition, in December 2024, we issued and sold to certain institutional investors an aggregate of (i) 2,793,562 shares of common stock and (ii) warrants to purchase up to 3,491,953 shares of common stock (the "2024 Warrants"). In June 2025, we issued and sold to certain institutional investors warrants to purchase up to 34,999,999 shares of common stock (the "2025 PIPE Warrants"), and we also issued a warrant to purchase up to 16,000,000 shares of common stock (the "RemeGen Warrant") as partial consideration for the Telitacicept License Agreement to a subsidiary of RemeGen. As of December 31, 2025, the 2024 Warrants and RemeGen Warrant remained outstanding and unexercised, and 29,920,359 of the 2025 PIPE

Warrants remain outstanding and unexercised. In addition, as of December 31, 2025, we have outstanding options to purchase 6,608,266 shares of common stock and 186,325 restricted stock units, and we have 2,539,361 shares of common stock available for future issuance under our 2023 Inducement Plan, 433,804 shares of common stock available for future issuance under our Amended and Restated 2021 Equity Incentive Plan and 115,932 shares of common stock available for issuance under our Employee Stock Purchase Plan. Should all of these shares be issued, you would experience substantial dilution in ownership of our common stock.

If we raise funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or we may have to grant licenses on terms that may not be favorable to us or commit to providing us with future payment streams. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Market volatility may further adversely impact our ability to access capital as and when needed.

We have a limited operating history, have not yet completed any clinical trials and have no history of commercializing products, which may make it difficult to evaluate the success of our business to date and to assess our future viability.

We are a clinical-stage company with no products approved for marketing. We were founded in December 2015 and commenced operations in February 2016. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our platform and technology, identifying product candidates and undertaking studies. Prior to our in-license of telitacicept in June 2025, our efforts were focused on developing engineered hematopoietic stem cell transplants, chimeric antigen receptor-T cell therapies and antibody drug conjugates for the treatment of acute myeloid leukemia. We are currently developing telitacicept in a global Phase 3 clinical trials for the treatment of gMG and SjD. The risk of failure for these activities is high. We have not yet demonstrated an ability to successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial-scale product or arrange for a third party to do so on our behalf or conduct sales and marketing activities necessary for successful commercialization. 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.

Our limited operating history may make it difficult to evaluate our technology and industry and predict our future performance. Our short history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We expect to encounter risks and difficulties frequently experienced by early stage companies in new and rapidly evolving fields. If we do not address these risks and difficulties successfully, our business could suffer.

In addition, we may encounter other unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We have never generated revenue from product sales and may never become profitable.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborators, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, product candidates. We do not anticipate generating revenues from product sales for the next several years, if ever. Our ability to generate future revenue from product sales depends heavily on our, or our current or future collaborators', ability to successfully:

- advance and complete clinical trials of our product candidates, including telitacicept;
- complete research and preclinical and clinical development of any other product candidates we may identify;

- seek and obtain regulatory and marketing approvals for any product candidates for which we complete clinical trials;
- launch and commercialize any product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualify for coverage and adequate reimbursement by government and third-party payors for any product candidates for which we obtain regulatory and marketing approval;
- establish and maintain supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for any product candidates for which we obtain regulatory and marketing approval;
- obtain market acceptance of product candidates as viable treatment options;
- address competing technological and market developments;
- implement internal systems and infrastructure, as needed;
- negotiate favorable terms in any collaboration, licensing or other arrangements into which we may enter and perform our obligations in such arrangements;
- maintain, protect, enforce, defend and expand our portfolio of intellectual property rights, including patents, trade secrets and know-how, in the United States and internationally;
- avoid and defend against third-party interference, infringement and other intellectual property claims in the United States and internationally; and
- attract, hire and retain qualified personnel.

Even if one or more of the product candidates we develop are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the U.S. Food and Drug Administration (the "FDA"), the European Medicines Agency (the "EMA") or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved product candidates, we may not become profitable and may need to obtain additional funding to continue operations.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause stockholders to lose all or part of their investment in us.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes to offset taxable income or taxes may be limited.

As of December 31, 2025, we had gross federal net operating loss carryforwards of $411.7 million including $409.8 million that had an indefinite carryforward period and $1.9 million that were subject to expiration at various dates through 2037. Furthermore, we have state and local net operating loss carryforwards of $389.4 million which will expire at various dates through 2045. Portions of these net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act (the "Tax Act"), as modified by the Coronavirus Aid, Relief, and Economic Security (the "CARES Act") U.S. federal net operating losses incurred in taxable years beginning after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal net operating losses in taxable years beginning after December 31, 2020, may be limited. It is uncertain how various states will respond to the Tax Act and the CARES Act. For state income tax purposes, there may be periods during which the use of net operating loss carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the "Code"), and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is 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 net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. The completion of our initial public offering, together with private placements and other transactions that have occurred since our inception, may trigger such an ownership change pursuant to Section 382 of the Code. We have not yet completed a Section 382 analysis, and therefore, there can be no assurances that our net operating losses are not already limited. We may experience ownership changes as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our net operating loss carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations. There is a full valuation allowance for net deferred tax assets, including net operating loss carryforwards.

Risks Related to Development, Manufacturing and Commercialization

We are substantially dependent on the success of our lead product candidate, telitacicept. If we are unable to complete development of, obtain approval for and commercialize telitacicept in a timely manner, our business will be harmed.

Our future success is dependent on our ability to timely advance and complete clinical trials, obtain marketing approval for and successfully commercialize our lead product candidate, telitacicept. We are investing significant efforts and financial resources in the research and development of telitacicept. Telitacicept is currently in global Phase 3 clinical trials for the treatment of gMG and SjD, and will require additional clinical development, evaluation of clinical and manufacturing activities, marketing approval from government regulators, substantial investment and significant marketing efforts before we can generate any revenues from product sales. We are not permitted to market or promote telitacicept or any other product candidate before we receive marketing approval from the FDA and comparable foreign regulatory authorities, and we may never receive such marketing approvals.

The success of telitacicept will depend on several factors, including the following:

- the approval or acceptance to initiate clinical trials by the applicable regulatory authorities in each country where we plan to conduct clinical trials;
- the acceptance of individual investigational review boards ("IRBs") and scientific review committees at each clinical trial site as to the adequacy of the preclinical data package to support clinical development of telitacicept and their overall general agreement with the use of telitacicept in the intended patient population in the intended manner;
- the willingness of clinical investigators to place patients in clinical trials, and the willingness of patients to enroll in clinical trials;
- the successful and timely completion of the global Phase 3 clinical trials of telitacicept in gMG and SjD;
- the initiation and successful patient enrollment and completion of additional clinical trials of telitacicept on a timely basis;
- maintaining and establishing relationships with contract research organizations ("CROs") and clinical sites for the clinical development of these programs both in the United States and internationally;
- the frequency and severity of adverse events in the clinical trials;
- the results of clinical trials conducted by third parties, including RemeGen, in autoimmune disorders if such trials result in changes to the standard of care for autoimmune disorders;
- the efficacy, safety and tolerability profiles that are satisfactory to the FDA, the EMA or any comparable foreign regulatory authority for marketing approval;
- the timely receipt of marketing approvals for our programs from applicable regulatory authorities;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- the maintenance of existing or the establishment of new supply arrangements with third-party suppliers and manufacturers for clinical development of our programs;

- our ability to obtain and maintain arrangements with third-party manufacturers to produce finished products that are appropriate for commercial sale of our programs, if approved;
- obtaining and maintaining patent protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- the protection of our rights in our intellectual property portfolio;
- the successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by patients, the medical community and third-party payors;
- our ability to obtain coverage and adequate reimbursement from third-party payors for our products and patients' willingness to pay out-of-pocket in the absence of such coverage and adequate reimbursement; and
- our ability to compete with other treatments.

We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize telitacicept, which would materially harm our business. If we do not receive marketing approval for telitacicept, we may not be able to continue our operations.

We may derive results and data for telitacicept from clinical trials conducted by RemeGen in China; our access to the clinical results and data may be limited and there is no assurance that the clinical data from any such trials will be accepted or considered by the FDA, or other comparable regulatory authorities.

RemeGen has received regulatory approval in China for telitacicept for the treatment of gMG, RA and SLE, and RemeGen is developing telitacicept in clinical trials in China in additional indications. While these trials may provide us with clinical data that can inform our future development strategy, we do not have control over the protocols, administration, or conduct of the trials or their compliance with regulatory requirements. There is also no assurance that the clinical data from any such clinical trials will be accepted or considered by the FDA or other comparable regulatory authorities. We have no control over the conduct and timing of, and communications with the National Medical Products Administration ("NMPA") or other foreign regulatory agencies in Greater China with respect to, the trials that RemeGen is conducting for telitacicept. Any data integrity issues or patient safety issues arising out of any of these trials would be beyond our control, yet could adversely affect our reputation and damage the clinical and commercial prospects for our product candidates.

We are dependent on third parties accurately generating and reporting data related to our product candidates, and their conduct could adversely affect our business.

We have and may in the future acquire or in-license our product candidates at various stages of development. For example, we in-licensed telitacicept from RemeGen. Our assumptions about the potential of telitacicept are partially based on data generated from preclinical studies and clinical trials conducted by RemeGen. We are dependent on RemeGen having conducted its research and development in accordance with the applicable protocols, informed consent, legal and regulatory requirements, and scientific standards, having accurately reported the results of all studies conducted, and having correctly collected the data from these studies. If these activities were not compliant, accurate or correct, the clinical development, regulatory approval or commercialization of telitacicept will be adversely affected.

Additionally, in cases where third parties conduct clinical trials using our product candidates through partnership or licensing agreements, we face additional risks related to the conduct and outcome of those trials that are outside of our direct control. For example, issues such as poor data integrity, safety concerns, protocol violations, or failure to meet endpoints in these third-party trials could adversely impact the development timeline and regulatory approval process for those product candidates in other indications or territories, require additional

studies, create negative market perception affecting future commercial potential, impact our ability to pursue strategic alternatives for such product candidates, or result in increased regulatory scrutiny across our programs.

Our clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could increase our costs or necessitate the abandonment or limitation of the development of our product candidates.

If our product candidates are associated with side effects in clinical trials or have characteristics that are unexpected, our costs could increase or we may need to abandon their development or limit development to more narrow uses in which the side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. The FDA or an IRB may also require that we suspend, discontinue, or limit our clinical trials based on safety information. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further development of the product candidate.

Before we can obtain marketing approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication, and failures can occur at any stage of testing. Clinical trials often fail to demonstrate safety and efficacy of the product candidate studied for the target indication.

Additionally, if we or others identify undesirable side effects caused by our product candidates, a number of potentially significant negative consequences could result, including:

- we may need to abandon the development or limit the further development of our product candidates, including in various populations and for certain indications;
- regulatory authorities may withdraw approval to market such product;
- regulatory authorities may require additional warnings on the labels;
- a medication guide outlining the risks of such side effects for distribution to patients may be required;
- we could be sued and held liable for harm caused to patients; and
- our reputation and physician or patient acceptance of our product candidates, if approved, may suffer.

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

From time to time, we may publicly disclose interim, topline or preliminary data from our clinical trials, which are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a full analysis of all data related to the particular trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. In addition, we may report preliminary analyses of only certain endpoints rather than all endpoints. As a result, the interim, topline or preliminary results that we report may differ from future results of the same trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim, topline and preliminary data should be viewed with caution until the final data are available. We may also disclose interim data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as subject enrollment continues and more subject data become available. Adverse differences between interim, topline or preliminary data and final data could significantly harm our reputation and business prospects. Further, disclosure of

interim, topline or preliminary data by us or by our competitors could result in volatility in the price of our common stock.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the potential of the particular program, the likelihood of marketing approval or commercialization of the particular product candidate, any approved product, and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is derived from information that is typically extensive, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular program, product candidate or our business.

If the interim, topline or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to further develop, obtain marketing approval for and/or commercialize our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Success in preclinical studies or clinical trials may not be indicative of results in future clinical trials, particularly for clinical trials that involve only a small number of patients.

Results from preclinical studies are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. This risk is heightened when clinical trials involve only a small number of patients, which makes it difficult to predict whether early results from these trials will be indicative of the final results of the trials or be replicated in future trials. Further, success in preclinical studies and earlier stage clinical trials of telitacicept conducted by RemeGen, or the fact that telitacicept has received marketing approval in China in multiple indications, does not ensure that later clinical trials conducted by us will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. For example, the results of RemeGen's Phase 3 clinical trials of telitacicept in gMG and SjD may not be replicated in our ongoing global Phase 3 clinical trials of telitacicept in gMG and SjD due to a variety of factors, including differences in trial design, patient demographics and enrollment and placebo rates. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. This failure to establish sufficient efficacy and safety could cause us to abandon clinical development of our product candidates.

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 managerial resources, we focus on product candidates and research programs that we identify for specific indications among many potential options. 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 product candidates and research and development programs for specific indications may not yield any commercially viable products. For example, in 2025 we wound down our then-ongoing clinical and manufacturing operations and clinical trials and, after a strategic evaluation process, in June 2025 we entered into a license agreement with RemeGen related to telitacicept and focused our operations on the development of that clinical program. Further, 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. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects.

Even if a product candidate receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

The commercial success of our product candidates, if approved, will depend upon their degree of market acceptance by physicians, patients, third-party payors and others in the medical community. Even if any product candidate we develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. The degree of market acceptance of any product candidate we develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidate as demonstrated in clinical trials;
- the efficacy and safety of other products that are used in combination or in sequence with our product;
- the potential and perceived advantages of our product candidates compared to alternative treatments;
- the limitation to our targeted patient population and limitations or warnings contained in approved labeling by the FDA or other regulatory authorities;
- the ability to offer our products for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA, the EMA or other regulatory authorities;
- the willingness of the target patient population to try novel biologics and of physicians to prescribe these treatments, as well as their willingness to accept an intervention that involves the alteration of the patient's gene;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- the strength of marketing and distribution support;
- availability of third-party coverage and sufficiency of reimbursement; and
- the prevalence and severity of any side effects.

Even if a product candidate is approved, such product may not achieve an adequate level of acceptance, we may not generate significant product revenues, and we may not become profitable.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing those product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have limited experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing and commercial support infrastructure to sell, or participate in sales activities with our collaborators for, some of our product candidates if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or

reimbursement specialists is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our commercialization personnel.

Factors that may inhibit our efforts to commercialize our product candidates on our own include:

- our inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to obtain access to physicians or educate adequate numbers of physicians on the benefits of prescribing any future products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payors;
- restricted or closed distribution channels that make it difficult to distribute our product candidates to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

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

We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize product candidates, if approved.

The development and commercialization of new drug and biologic products is highly competitive. We will face competition with respect to our product candidates that we develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Competition in the autoimmune field is intense and involves multiple monoclonal antibodies (mAbs), other biologics and small molecules either already marketed or in development by many different companies including large pharmaceutical companies such as Alexion (Solaris and Ultomiris/Myasthenia Gravis), Amgen, Inc. (Uplinza/Myasthenia Gravis), Argenx (VYVGART and VYVGART HYTRULO/Myasthenia Gravis), UCB (Restage/Myasthenia Gravis), Johnson & Johnson (Imaavy/Myasthenia Gravis), GlaxoSmithKline plc (Benlysta/lupus), F. Hoffman-La Roche AG (Rituxan/often used off label).

We face and expect to continue to face intense competition from other biopharmaceutical companies, who have launched or are developing products for the treatment of gMG and other autoimmune diseases. Competition for other indications is also fierce, with significant development in almost all of the indications we may develop for our product candidates. Novartis AG, CSL Behring, Grifols, S.A., Curavac, Inc., Takeda Pharmaceutical Co Ltd, Immunovant, Inc., Cartesian Therapeutics, Inc., Amgen, Kyverna, Dianthus, and Regeneron Pharmaceuticals

Inc./Alnylam Pharmaceuticals, Inc., among others, are developing drugs that may have utility for the treatment of myasthenia gravis (MG) or Sjogren's Disease (SjD)

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates. This may include other types of therapies, such as small molecule, CAR-T, antibody and/or protein therapies.

Many of our current or potential competitors, either alone or with their collaboration partners, may 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. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. 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 registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize product candidates that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our product candidates or that would render our product candidates obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their product candidates more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates against competitors.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for our product candidates, if approved.

Negative developments in the field of protein-based therapies, including in particular fusion protein therapies, or approved therapies or therapies in development for the treatment of B cell-mediated autoimmune diseases, could damage public perception of our product candidate and negatively affect our business.

The commercial success of our product candidate will depend in part on public acceptance of the use of fusion protein therapies constructed by joining two or more domains encoded by different genes, as well as protein-based therapies more generally, including in particular approved therapies or therapies that are in development for the treatment of B cell-mediated autoimmune diseases. Telitacicept is a novel fusion protein in development for treating B cell mediated autoimmune diseases that inhibits both BLyS (BAFF) and APRIL. Adverse events in post marketing use in any country in any approved indication or off label use, in clinical trials of our product candidate or in clinical trials of others developing similar product candidates, including RemeGen, and the resulting publicity, as well as any other negative developments that may occur in the future, including in connection with competitors' therapies, could result in a decrease in demand for our product candidate. These events could also result in the suspension, discontinuation, or clinical hold of, or modifications to, our clinical trials. Our product candidate may not be accepted by the general public or the medical community and potential clinical trial subjects may be discouraged from enrolling in our clinical trials or early terminated from the clinical trials. As a result, we may not be able to continue, or may be delayed in conducting, our development programs.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability exposure related to the testing in human clinical trials of our product candidates and will face an even greater risk if we commercially sell any products that we may develop. For example, we may be sued if our product candidates cause, or are perceived to cause, injury or are found to be otherwise unsuitable during clinical trials, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the

product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- the inability to commercialize any products that we may develop;
- decreased demand for our product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant time and costs to defend the related litigation;
- substantial monetary awards to trial participants or patients; and
- loss of revenue.

Insurance coverage is also increasingly expensive and we may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Fusion protein therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of telitacicept or other product candidates or otherwise harm our business.

The manufacture of fusion proteins, such as telitacicept, is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for clinical trials and commercial products for telitacicept, if approved, or any fusion protein product that we may develop in the future. Additionally, because biologic products are complex, the manufacture of such product candidates is more difficult and costly. We may not be able to have such products reliably manufactured in accordance with the applicable regulatory requirements in sufficient quantities to support our development programs and, if ultimately approved, commercial supply.

There are a limited number of contract manufacturers who specialize in the manufacture of biologic products and those that do may still be developing appropriate processes, controls and facilities for large-scale production. While we believe that there will be sufficient sources of supply that can satisfy our clinical and commercial requirements, we cannot be certain that we will be able to identify and establish additional relationships with such sources, if necessary, in a timely manner or at all, and what the terms and costs of such new arrangements would be, or that such suppliers would be able to supply our potential commercial needs. Furthermore, in the event our primary manufacturer cannot meet our needs, any switch to an alternative manufacturer, if available, would result in a significant delay, would require FDA approval, and cause material additional costs.

The manufacturers of biologic products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our contract manufacturing organizations to adhere to or document compliance with such regulatory requirements could lead to a delay or interruption in the availability of drug product for clinical trials or commercial use, among other consequences. If we or our manufacturers fail to comply with the FDA, EMA, or other regulatory authorities, it could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, clinical holds or termination of clinical trials, Form 483s, warning or untitled letters, regulatory communications warning the public about safety issues with a product, import or export refusals, license revocation, seizures, detentions, or recalls of product candidates or product, operating restrictions, criminal prosecutions or debarment, suits under the civil False Claims Act, corporate integrity agreements, or consent decrees any of which could significantly and adversely affect supplies of our product candidates and our business, financial conditions and results of operations could be materially adversely affected.

Our current dependence upon others for the manufacture of our product candidates may also adversely affect our business, results of operations, financial condition, and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

If we or any contract manufacturers and suppliers that we engage fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state and local environmental, health and safety laws, regulations and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our product development and research efforts. In addition, we cannot eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, we may incur substantial costs to comply with current or future environmental, health and safety laws, regulations and permitting requirements. These current or future laws, regulations and permitting requirements may impair our research, development or production efforts. Failure to comply with these laws, regulations and permitting requirements also may result in substantial fines, penalties or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Regulatory Review

If clinical trials of any of our product candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidates.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must complete extensive clinical trials to demonstrate the safety and efficacy in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results.

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates.

We and our collaborators, if any, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize any product candidates, including:

- delays in reaching a consensus with regulators on clinical development plans, trial designs or regulatory approval data packages;
- regulators, IRBs, independent ethics committees or scientific review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- delays in reaching or failing to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective CROs, and clinical trial sites;
- clinical trials of product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development or research programs;
- difficulty in designing well-controlled clinical trials due to ethical considerations which may render it inappropriate to conduct a trial with a control arm that can be effectively compared to a treatment arm;
- difficulty in designing clinical trials and selecting endpoints for diseases that have not been well-studied and for which the natural history and course of the disease is poorly understood;
- the number of patients required for clinical trials may be larger than we anticipate; enrollment of suitable participants in these clinical trials, which may be particularly challenging for some of the rare diseases we are targeting in our most advanced programs, may be delayed or slower than we anticipate; or patients may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, IRBs or independent ethics committees may require that we or our investigators suspend or terminate clinical research or clinical trials for various reasons, including noncompliance with regulatory requirements, a finding of undesirable side effects or other unexpected characteristics, or that the participants are being exposed to unacceptable health risks or after an inspection of our clinical trial operations or trial sites;
- the cost of clinical trials may be greater than we anticipate;
- the supply or quality of product candidates or other materials necessary to conduct clinical trials may be insufficient or inadequate, including as a result of delays in the testing, validation, manufacturing and delivery of product candidates to the clinical sites by us or by third parties with whom we have contracted to perform certain of those functions;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with product candidates that are viewed to outweigh their potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- disruption in the supply or availability of drug product; and
- changes in the standard of care treatment guidelines.

If we or our collaborators are required to conduct additional clinical trials or other testing of product candidates beyond those that we currently contemplate, if we or our collaborators are unable to successfully complete clinical trials or other testing of product candidates, or if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we or our collaborators may:

- be delayed in obtaining marketing approval for any such product candidates or not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw or suspend their approval of the product or impose restrictions on its distribution in the form of a REMS or through modification to an existing REMS;
- be sued; or
- experience damage to our reputation.

Product development costs will also increase if we or our collaborators experience delays in clinical trials or other testing or in obtaining marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize product candidates, could allow our competitors to bring products to market before we do and could impair our ability to successfully commercialize product candidates, any of which may harm our business, financial condition, results of operations and prospects.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize telitacicept or any other product candidate we may develop in the United States or any other jurisdiction, and any such approval may be for a more narrow indication than we seek.

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a REMS. These regulatory authorities may require labeling that includes precautions or contra-indications with respect to conditions of use, or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially adversely affect our business, financial condition, results of operations and prospects.

Marketing approval by the FDA in the United States, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product candidate testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which

could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our product candidates in those countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our product candidates will be unrealized.

If we experience significant delays or difficulties in the enrollment or retention of patients in clinical trials, the cost of developing product candidates could increase and our receipt of necessary regulatory approvals could be delayed or prevented.

Patient enrollment is a significant factor in the timing of clinical trials. The timing of our clinical trials depends, in part, on the speed at which we can recruit patients to participate in our trials. We or our collaborators may not be able to advance clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, the EMA or other analogous regulatory authorities outside the United States, or as needed to provide appropriate statistical power for a given trial. Patients may be unwilling to participate in our clinical trials because of negative publicity from adverse events related to the biotechnology field, RemeGen's telitacicept products or clinical trials, competitive clinical trials for similar patient populations, clinical trials in competing products or for other reasons. As a result, the timeline for recruiting patients, conducting trials and obtaining regulatory approval of product candidates could be delayed. In addition, if an unexpected number of patients drop out from a trial early, it would negatively impact the integrity of the trial results.

Patient enrollment and retention is also affected by other factors, including:

- severity of the disease under investigation;
- size of the patient population and process for identifying patients;
- design of the trial protocol;
- ability to obtain and maintain patient informed consent;
- risk that enrolled patients will drop out before completion of the trial, including due to side effects or characteristics that are unrelated to our product candidate;
- eligibility and exclusion criteria for the trial in question;
- perceived risks and benefits of the product candidate under trial;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- ability to monitor patients adequately during and after treatment;
- new therapies that are or become approved and available to the same patient population; and
- changes in standard of care treatment guidelines.

Significant enrollment delays or poor patient retention 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. If we or our collaborators have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, or have difficulty retaining patients, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Our Relationships with Third Parties

We rely on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing.

We rely on third parties, such as CROs, clinical data management organizations, and clinical investigators, to conduct our clinical trials. Any of these third parties may terminate their engagements with us at any time under certain criteria. If we need to enter into alternative arrangements, it may delay our product development activities.

Our reliance on these third parties for clinical activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA, EMA and other regulatory authorities require us to comply with standards, commonly referred to as Good Clinical Practices, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. In the United States, we also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Although we intend to design the future clinical trials for our product candidates, CROs will conduct some or all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct clinical trials will also result in less direct control over the management of data developed through clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CROs and other third parties do not perform clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of our product candidates may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates, or our development programs may be materially and irreversibly harmed. If we are unable to rely on preclinical and clinical data collected by our CROs and other third parties, we could be required to repeat, extend the duration of or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of our product candidates.

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

We will continue to contract with third parties for the manufacture and supply of materials for development of our product candidates, advancement of our current and future clinical trials, and potential commercialization of our product candidates. This increases the risk that we will not have sufficient quantities and quality of such materials for the development of our product candidates, or that such supply will not be available to us at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We will rely on third-party manufacturers, including certain single source suppliers, for the manufacture and supply of materials for development of our product candidates and advancement of our current clinical trial, and expect to continue to do so for future clinical testing and for commercial supply of our product candidates for which we or any future collaborators obtain marketing approval. We do not have a long-term agreement with many of these third-party manufacturers or suppliers. We may be unable to establish any agreements with third-party manufacturers or suppliers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers or suppliers, reliance on third-party manufacturers entails additional risks, including:

- the possible breach of the manufacturing or supply agreement by the third party;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and
- reliance on the third party for regulatory compliance, quality assurance, safety and pharmacovigilance and related reporting.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers or suppliers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products and harm our business, financial condition, results of operations and prospects.

Our product candidates may compete with other product candidates and products for access to manufacturing facilities and other supplies. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Also, prior to the approval of our product candidates, we would need to identify a contract manufacturer that could produce our products at a commercial scale and that could successfully complete FDA pre-approval inspection and inspections by other health authorities. Agreements with such manufacturers or suppliers may not be available to us at the time we would need to have that capability and capacity.

Any performance failure on the part of our existing or future manufacturers or suppliers, or any decision by a manufacturer or supplier to remove its products from the market or restrict access to its products, could delay clinical development or marketing approval. Although we believe that there are several potential alternative manufacturers who could replace our contract manufacturers, we may incur added costs and delays in identifying and qualifying any such replacement.

Our current and anticipated future dependence upon others for the manufacture of our product candidates and the materials used in our clinical trials may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

We have and may enter into collaborations with third parties for the research, development and commercialization of our product candidates. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We have and may seek third-party collaborators for the research, development and commercialization of certain our product candidates. If we enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of collaborations that we have entered into or may enter into in the future.

Collaborations involving our current or future product candidates or research programs pose numerous risks to us, including the following:

- Collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations.
- Collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities.
- Collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing.
- Collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours.
- Collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such products.
- Collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation.
- Disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources.
- We may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control.
- Collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.
- Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished or terminated.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it

more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this "Risk Factors" section apply to the activities of our collaborators.

These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of several factors. If we license rights to our product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of the product candidates, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We would face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA, the EMA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

We may also be restricted under collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to develop product candidates or bring them to market and generate product revenue.

Risks Related to Our Intellectual Property

We are highly dependent on intellectual property licensed from third parties and termination of these licenses could result in the loss of significant rights, which would harm our business.

In June 2025, we entered into a license agreement with RemeGen granting us an exclusive (even as to RemeGen) license under RemeGen's patents and know-how to exploit, develop and commercialize telitacicept in all

territories other than Greater China, with the right to grant sublicenses, and a non-exclusive license to manufacture the telitacicept worldwide for use in the licensed territory.

We are dependent on the patents, know-how and proprietary technology licensed from RemeGen for the development and, if approved, commercialization of telitacicept. Any termination of this license, or a finding that such intellectual property lacks legal effect, could result in the loss of significant rights and could harm our ability to commercialize our product candidate.

The RemeGen license agreement imposes certain obligations on us, and non-compliance with such obligations may result in termination of the license agreement or in legal and financial consequences. If RemeGen terminates the license agreement, we may not be able to develop, commercialize or sell our product candidate covered by the agreement. Such an occurrence could materially adversely affect the value of the product candidate being developed under the agreement or using rights granted under such agreement. Termination of our license agreement or reduction or elimination of our rights thereunder may result in our having to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all, which may mean we are unable to develop, commercialize or sell the affected product candidate or may cause us to lose our rights under the agreement.

In addition, our licensors may make decisions in prosecuting, maintaining, enforcing and defending any licensed intellectual property rights, for example, any licensed patents or patent applications, that may not be in our best interest. Moreover, if our licensors take any action with respect to any licensed intellectual property rights, for example, any licensed patents or patent applications, that results in a successful challenge to the licensed intellectual property by a third party, such patents may be invalidated or held to be unenforceable, and we may lose our rights under such patents, which could materially harm our business.

Further, license agreements under which we license intellectual property from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. Accordingly, disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including those relating to:

- the scope of rights, if any, granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the license agreement;
- whether our licensor or its licensor had the right to grant the license agreement;
- whether third parties are entitled to compensation or equitable relief, such as an injunction, for our use of the intellectual property without their authorization;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- whether we are complying with our obligations with respect to the use of the licensed technology in relation to our development and commercialization of product candidates;
- our involvement in the prosecution and enforcement of the licensed patents and our licensors' overall patent prosecution and enforcement strategy;
- the allocation of ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and by us and any future partners or collaborators; and
- the amounts of royalties, milestones or other payments due under the license agreement.

The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, or are insufficient to provide us the necessary rights to use the intellectual property, we may be unable to successfully develop and commercialize the affected product candidates.

If we or any of our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer. Any disputes with our licensors or any termination of the licenses on which we depend could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Our commercial success depends in large part on our ability to obtain, maintain and protect intellectual property rights through patents, trademarks and trade secrets in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode, negate or preempt any competitive advantage we may have, which could harm our business and ability to achieve profitability.

To protect our proprietary position, we own and have in-licensed certain intellectual property rights, including certain issued patents and patent applications, and have filed and may file provisional and non-provisional patent applications in the United States or abroad related to our product candidates that are important to our business. Provisional patent applications are not eligible to become issued patents until, among other things, we file a non-provisional patent application within 12 months of the filing of one or more of our related provisional patent applications. If we do not timely file non-provisional patent applications, we may lose our priority date with respect to our provisional patent applications and any patent protection on the inventions disclosed in our provisional patent applications. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether any such patent applications will result in the issuance of patents that provide us with any competitive advantage. Moreover, the patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

The patent application, prosecution and enforcement processes are subject to numerous risks and uncertainties, and there can be no assurance that we, our licensors, or any of our current or future collaborators will be successful in protecting our product candidates by obtaining, defending and/or asserting patent rights. These risks and uncertainties include the following:

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

In some instances, agreements through which we license intellectual property rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented, how claims are amended, and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. While we have the first right to prosecute patents and patent applications we license under our license agreement with RemeGen in the licensed territory, RemeGen retains the right to prosecute these patents and patent applications in the remaining territory, and therefore we cannot guarantee that these patents and applications will be prosecuted or maintained in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents.

Moreover, some of our in-licensed patents and patent applications may be, and some of our future owned and licensed patents may be, co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us.

The patent protection we obtain for our product candidates may not be sufficient to provide us with any competitive advantage or our patents may be challenged.

Our owned and licensed patents and pending patent applications, if issued, may not provide us with any meaningful protection or may not prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, a third party may develop a competitive product that provides benefits similar to one or more of our product candidates but falls outside the scope of our patent protection or license rights. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Currently, a significant portion of our patents and patent applications are in-licensed, though similar risks would apply to any patents or patent applications that we now own or may own or in-license in the future.

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

In addition, the determination of patent rights with respect to clinical compositions of matter and treatment methods commonly involves complex legal and factual questions, which are dependent upon the current legal and intellectual property context, extant legal precedent and interpretations of the law by individuals. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are characterized by uncertainty.

Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, patent laws in various jurisdictions, including significant commercial markets such as Europe, restrict the patentability of methods of treatment of the human body more than U.S. law does. If these changes were to occur, they could have a material adverse effect on our ability to generate revenue.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for

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

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our owned and licensed patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party submission of prior art to the USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivation proceedings, *ex parte* reexaminations, *inter partes* review, supplemental examinations, or interference proceedings or challenges in district court, in the United States or in various foreign patent offices, including both national and regional, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of the patent or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, or in denial of the patent application or loss or reduction in the scope of one or more claims of the patent application, any of which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

Issued patents that we have or may obtain or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products, for example, by submitting a Section 351(k) Biologics License Application (”BLA”) to the FDA, or pursue similar strategies in the United States or other jurisdictions, in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Other parties have developed or may develop technologies that may be related to or competitive with our approach, and may have filed or may file patent applications and may have been issued or may be issued patents with claims that overlap or conflict with our patent applications, either by claiming the same materials, formulations or methods, or by claiming subject matter that could dominate our patent position. In addition, certain parts or all of the patent portfolios licensed to us are, or may be, licensed to third parties and such third parties may have or may obtain certain enforcement rights. If the scope of the patent protection we or our licensors obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep

any competitive advantage. We cannot provide any assurances that any of our licensed patents have, or that any of our pending owned or licensed patent applications that mature into issued patents will include, claims with a scope sufficient to protect our product candidates or otherwise provide any competitive advantage, nor can we provide any assurance that our licenses will remain in force.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position may be harmed.

In addition to the protection afforded by patents, we rely upon trade secret protection, know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, scientific advisors, employees and consultants and invention assignment agreements with our consultants and employees. However, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property rights under these agreements may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. In addition, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements despite the existence of confidentiality agreements and other contractual restrictions. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, scientific advisors, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim against a third party that illegally obtained and is using our trade secrets, like patent litigation, is expensive and time-consuming and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing or unwilling to protect trade secrets. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Moreover, our trade secrets could otherwise become known or be independently discovered by our competitors or other third parties. Competitors and other third parties could attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

We may not be successful in acquiring or in-licensing necessary rights to key technologies underlying our product candidates.

We currently have rights to intellectual property, through licenses from third parties, to develop our product candidates, and we expect to seek to expand our intellectual property footprint related to our product candidate pipeline in part by in-licensing the rights to key technologies. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to develop additional product candidates and technologies. Although we have succeeded in licensing technologies from third party licensors, including RemeGen, in the past, we can give no assurance that we will be able to in-license or acquire the rights to other technologies relevant to our product candidates from third parties on acceptable terms or at all.

In order to market our product candidates, we may find it necessary or prudent to obtain licenses from such third party intellectual property holders. However, it may be unclear who owns the rights to intellectual property we wish to obtain, or we may be unable to secure such licenses or otherwise acquire or in-license intellectual property rights from third parties that we identify as necessary for our product candidates and technology we employ. We currently conduct our clinical trials under 35 U.S.C. § 271(e)(1), which provides a safe harbor from patent

infringement for uses of patented technology reasonably related to the development and submission of information under a federal law which regulates the manufacture, use, or sale of drugs.

The licensing or acquisition of third party intellectual property rights is a highly competitive area, and other companies may pursue strategies to license or acquire third party intellectual property rights that we may consider attractive or necessary. Such companies may have a competitive advantage over us, e.g., due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Even if we were able to obtain such a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

Third-party claims of intellectual property infringement, misappropriation or other violations may prevent or delay our product discovery and development efforts and have a material adverse effect on our business.

Our commercial success depends in part on our avoiding infringement, misappropriation and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U.S. patent reform, new procedures including *inter partes* review and post grant review have been implemented. This reform will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates, and third parties may allege they have patent rights encompassing our product candidates, technologies or methods. Third parties may assert that we are employing their proprietary technology without authorization and may file patent infringement claims or lawsuits against us, and if we are found to infringe such third-party patents, we may be required to pay damages, cease commercialization of the infringing technology or obtain a license from such third parties, which may not be available on commercially reasonable terms or at all.

There may also be third-party patents of which we are currently unaware with patent rights to materials, formulations, methods of manufacture or methods of treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Further, we or our licensors may fail to identify even those relevant third-party patents that have issued or may incorrectly interpret the relevance, scope or expiration of such patents. The scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history. Our interpretation of the relevance or scope of a patent or a pending application may be incorrect. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, materials used in or formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our materials, formulations or methods, including without limitation, combination therapy or patient selection methods, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would involve a substantial diversion of employee resources from our business. We may not have sufficient resources to bring these actions to a successful conclusion, which may result in significant cost and may impede our inability to pursue any affected products or product candidates. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock.

In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market earlier than would otherwise have been the case, which would have a material adverse effect on our business.

Some intellectual property that we have in-licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers.

Any of the intellectual property rights that we have licensed or we may license in the future and that have been generated through the use of U.S. government funding are subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980 (the "Bayh-Dole Act"). These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any such intellectual property rights to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to such intellectual property rights if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. We cannot be certain that our current or future licensors will comply with the disclosure or reporting requirements of the Bayh-Dole Act at all times, or be able to rectify any lapse in compliance with these requirements.

In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to

manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects.

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

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the Supreme Court held that certain claims to DNA molecules are not patentable. In addition, the case *Amgen Inc. v. Sanofi* affects the way antibody claims are examined and litigated. While we do not believe that any of the patents owned or licensed by us will be found

invalid based on these decisions, we cannot predict how future decisions by the courts, the Congress or the USPTO may impact the value of our patents.

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

Filing, prosecuting, maintaining, defending and enforcing patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drugs and may export otherwise infringing drugs to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These drugs may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful.

Many countries have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license, which could adversely affect our business, financial condition, results of operations, and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest filing date of a non-provisional application to which the patent claims priority. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours.

If we do not obtain patent term extension and data exclusivity for patents related to any of our product candidates, our business may be materially harmed.

Depending upon the timing, duration and conditions of any FDA marketing approval of our product candidates, one or more 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, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product

development and the FDA regulatory review process. However, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. Only one patent per approved product can be extended, the extension cannot extend the total patent term beyond 14 years from approval and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for the applicable product candidate will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case, and our competitive position, business, financial condition, results of operations, and prospects could be materially harmed.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

We employ individuals who were previously employed at universities or other biopharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. We may also be subject to claims that patents and applications that we may file to protect inventions of our employees or consultants are rightfully owned by their former employers or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing would harm our business, financial condition, results of operations, and prospects.

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

Our registered or unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our business, financial condition, results of operations and growth prospects.

Intellectual property rights do not necessarily address all potential threats.

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

- Our product candidates may eventually become available in generic or biosimilar product forms;
- others may be able to make products that are similar to our product candidates or utilize similar technology but that are not covered by the claims of the patents that we license or own;

- we, or our current or future licensors might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or own;
- we, or our current or future licensors might not have been the first to file patent applications covering certain of our or their inventions;
- we, or our license partners or current or future collaborators, may fail to meet our obligations to the U.S. government regarding any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss or unenforceability of patent rights;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending, owned or licensed patent applications or those that we may own in the future will not lead to issued patents;
- it is possible that there are prior public disclosures that could invalidate our owned or in-licensed patents, or parts of our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our product candidates or technology similar to ours;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- it is possible that our pending owned or licensed patent applications or those that we may own or license in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- the laws of foreign countries may not protect our proprietary rights or the proprietary rights of license partners or current or future collaborators to the same extent as the laws of the United States;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes that design around our patents or become hostile to us or the patents or patent applications on which they are named as inventors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we may not develop additional proprietary technologies that are patentable;
- any product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could harm our business, financial condition, results of operations, and prospects.

Risks Related to Regulatory and Other Legal Compliance Matters

Failure to obtain marketing approval in foreign jurisdictions would prevent product candidates from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell product candidates in the European Union and other foreign jurisdictions, we or our third-party collaborators must obtain separate marketing approvals (a single one for the European Union) and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product candidate be approved for reimbursement before the product candidate can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any jurisdiction, which would materially impair our ability to generate revenue.

Even if we, or any collaborators we may have, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our product candidates could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our product candidates, which could materially impair our ability to generate revenue.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA, EMA, the Competent Authorities of the Member States of the European Union and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, facility registration and drug listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product. The FDA and other regulatory authorities may restrict the use of our products to certain specialists and/or institutions and require formal reporting and approval of a REMS program. Such restrictions or requirements could deter use of our products by certain individuals or institutions.

Accordingly, assuming we, or any of our collaborators, receive marketing approval for one or more product candidates, we, such collaborators and our and their contract manufacturers will continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we and such collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition and prospects.

Even if we receive regulatory approval for any product candidate, we will be subject to ongoing obligations and continued regulatory review, which may result in significant additional expense. Additionally, our product candidates for which we obtain marketing approval could be subject to labeling and other restrictions or withdrawal from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

If the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, testing, safety, efficacy, labeling, packaging, distribution, import, export, adverse event

reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMPs and GCPs for any clinical trials that we conduct post-approval, all of which may result in significant expense and limit our ability to commercialize such products. In addition, any regulatory approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

The FDA, the EMA, the Competent Authorities of the Member States of the European Union and other regulatory authorities closely regulate the post-approval marketing and promotion of products to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA, the Competent Authorities of the Member States of the European Union and other regulatory authorities impose stringent restrictions on manufacturers' communications regarding off-label use, and if we market our products for off-label use, we may be subject to enforcement action for off-label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice. While physicians may prescribe products for off-label uses as the FDA and other U.S. regulatory agencies do not regulate a physician's choice of drug treatment made in the physician's independent medical judgment, they do restrict promotional communications from companies or their sales force with respect to off-label uses of products for which marketing clearance has not been issued. Companies may only share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. Violation of the Federal Food, Product, and Cosmetic Act and other statutes, including the False Claims Act and equivalent legislation in other countries relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state and other countries' health care fraud and abuse laws and state consumer protection laws. Even if it is later determined we were not in violation of these laws, we may be faced with negative publicity, incur significant expenses defending our actions and have to divert significant management resources from other matters.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on the distribution or use of a product;
- requirements to conduct post-marketing clinical trials;
- receipt of warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenue;
- restrictions on future procurements with governmental authorities;
- suspension or withdrawal of marketing approvals;
- suspension of any ongoing clinical trials;
- refusal to permit the import or export of our products;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any of our product candidates, if approved, and adversely affect our business, financial condition, results of operations and prospects.

Moreover, the policies of the FDA and of comparable foreign regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. For example, the U.S. Supreme Court's June 2024 decision in Loper Bright Enterprises v. Raimondo overturned the longstanding Chevron doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The Loper decision could result in additional legal challenges to regulations and decisions issued by federal agencies, including the FDA, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the Loper decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability.

Disruptions at the FDA and other government agencies caused by funding shortages, layoffs, shifting policy priorities or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, layoffs, the FDA's ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new biologics or modifications to cleared or approved biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. Additionally, over the last several years, the U.S. government has shut down multiple times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If funding for the FDA is reduced, FDA priorities change, or a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

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

Healthcare providers and third-party payors 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 providers, third-party payors and customers may expose us to broadly applicable federal and state fraud and abuse, transparency, health data privacy, and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research as well as market, sell and distribute our products for which we obtain marketing approval.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Given the breadth of the laws and regulations and evolving government interpretations of the laws and regulations, governmental authorities may possibly conclude that our business practices, including certain of our advisory board arrangements with physicians, some of whom are compensated in the form of stock or stock options, may not comply with healthcare laws and regulations. If our operations are found to be in violation of any healthcare laws or any other federal or state government regulations that apply to us, we may be subject to significant penalties, including administrative, civil and criminal penalties, damages, fines, disgorgement, exclusion from participation in government health care programs, such as Medicare

and Medicaid, imprisonment, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations, and prospects.

Additionally, some state and local laws require certain regulatory licenses to manufacture or distribute our products commercially and/or the registration of pharmaceutical sales representatives in the jurisdiction. Further, 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 manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

Healthcare and other reform legislation, may increase the difficulty and cost for us and any collaborators we may have to obtain marketing approval of and commercialize our product candidates, if approved, and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been and continue to be ongoing efforts to implement legislative and regulatory changes regarding the healthcare system. Such changes could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates for which we obtain marketing approval. Although we cannot predict what healthcare or other reform efforts will be successful, such efforts may result in more rigorous coverage criteria, in additional downward pressure on the price that we, or our future collaborators, may receive for any approved products or in other consequences that may adversely affect our ability to achieve or maintain profitability.

Within the United States, the federal government and individual states have aggressively pursued healthcare reform, as evidenced by the passing of the ACA. The ACA substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for pharmaceutical products. There have been executive, judicial and Congressional challenges and amendments, to certain aspects of the ACA. For example, on July 4, 2025, the One Big Beautiful Bill Act (the "OBBBA") was signed into law, which narrowed access to ACA marketplace exchange enrollment and declined to extend the ACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired ACA subsidies.

The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at HHS, the FDA, the Centers for Medicare & Medicaid Services ("CMS") and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, the current administration has announced agreements with several pharmaceutical companies that require the drug manufacturers to offer, through a direct to consumer platform ("TrumpRx") U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the Make America Healthy Again ("MAHA") Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager ("PBM") payment methodologies, among other things. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In June 2024, the U.S. Supreme Court's Loper Bright decision greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations.

Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program.

The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish adequate coverage, reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability and adequacy of coverage and reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford prescription medications such as our product candidates, if approved. Our ability to achieve acceptable levels of coverage and reimbursement for products by governmental authorities, private health insurers and other organizations will have an effect on our ability to successfully commercialize our product candidates. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. Even if we obtain coverage for our product candidates by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. We cannot be sure that coverage and reimbursement in the United States, the EU or elsewhere will be available for our product candidates or any product that we may develop, and any reimbursement that may become available may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical, biopharmaceutical and biotechnology products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs or biologics when an equivalent generic drug, biosimilar or a less expensive therapy is available. It is possible that a third-party payor may consider our product candidates as substitutable and only offer to reimburse patients for the cost of the less expensive product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of existing third-party therapeutics may limit the amounts we will be able to charge for our product candidates. These payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in our product candidates. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our product candidates and may not be able to obtain a satisfactory financial return on our investment in the development of product candidates.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, third-party payors, including private and governmental payors, such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models in the United States for how private payors and other governmental payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. We cannot predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Further, HHS imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. HHS has also been empowered to negotiate the price of certain single-source biologics that have been on the market for at least eleven (11) years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis.

No uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that

coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases on short notice, and we believe that changes in these rules and regulations are likely.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe and other foreign jurisdictions have and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amounts that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products, and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of our product candidates due to the trend toward managed health care, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and biologics and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, consultants and commercial partners, and, if we commence clinical trials, our principal investigators. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the EMA and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA, the EMA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations, and prospects, including the imposition of significant fines or other sanctions.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.

We may be subject to numerous laws and regulations in each jurisdiction outside the United States in which we may operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act (the "FCPA") prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the

individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Similarly, the U.K. Bribery Act 2010 has extra-territorial effect for companies and individuals having a connection with the United Kingdom. The U.K. Bribery Act prohibits inducements both to public officials and private individuals and organizations. Compliance with the FCPA and the U.K. Bribery Act is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain product candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

We and the third parties with whom we work are subject to stringent and evolving privacy and information security laws, regulations, industry standards, policies, contractual obligations and other obligations related to privacy and information security. Our (or the third parties with whom we work) actual or perceived failure to comply with such obligations could adversely affect our business.

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 data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, sensitive third-party data, business plans, transactions, information about patients and clinical trial data (collectively, sensitive data).

Our data processing activities subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Actual or perceived failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment of company officials and public censure, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data 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, the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information

Technology for Economic and Clinical Health Act ("HITECH"), imposes specific requirements relating to the privacy, security, and transmission of individually identifiable protected health information. In addition, numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 (collectively, "CCPA"), applies to personal data of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. Although there are minimum revenue thresholds for companies to be subject to these laws and there are limited exemptions for clinical trial data under the CCPA and similar state comprehensive privacy laws, such laws may impact (possibly significantly) our business activities depending on how they are interpreted, should we become subject to the CCPA or such state comprehensive privacy laws in the future. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future. These developments may further complicate compliance efforts and increase legal risk and compliance costs for us and the third parties with whom we work.

Outside the United States, and increasing number of laws govern data privacy and security. For example, the European Union's General Data Protection Regulation ("EU GDPR)", the United Kingdom's GDPR ("UK GDPR")(collectively, "GDPR") and China's Personal Information Protection Law ("PIPL) impose strict requirements for processing personal data. For example, under the GDPR, companies may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR, 17.5 million pounds sterling under the UK GDPR or, in each case, 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

In the ordinary course of business, we transfer personal data from Europe and other jurisdictions to the United States or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the United Kingdom (UK) have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it generally believes are inadequate. Other jurisdictions may adopt or have already adopted similarly stringent data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA 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 to 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 data to the United States. If there is no lawful manner for us to transfer personal data from the EEA, the UK or other jurisdictions to the United States, 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 data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups.

Additionally, the U.S. Department of Justice issued a rule entitled Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restrictions on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered individuals (i.e., individuals and entities located in or controlled by individuals or entities located in those jurisdictions) that may impact certain business activities such as vendor engagements, employment of certain

individuals and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted.

Our employees and personnel use generative artificial intelligence ("AI") technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws and regulations regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and 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 bound by other contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We publish privacy policies and other statements concerning data privacy and security. Regulators in the United States are increasingly scrutinizing these statements, and if these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, 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, becoming increasingly stringent, and creating uncertainty. 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 us to devote significant resources, which may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf. We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we are unable to properly protect the privacy and security of sensitive data in our possession, we could be found to have breached our contracts. Further, if we fail to comply with applicable privacy and security laws, including applicable HIPAA privacy and security standards, we could face significant consequences, including but not limited to: government enforcement actions (e.g., administrative, civil and criminal penalties, investigations, audits, inspections, and similar); litigation (including class-action claims); additional reporting requirements and/or oversight; bans on processing personal data (including clinical trial data); and orders to destroy or not use personal data. In addition, our ongoing efforts to comply with evolving privacy and data security laws and regulations have been and may in the future be costly and require ongoing modifications to our policies, procedures and systems. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims 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.

Risks Related to Employee Matters, Managing Growth and Information Technology

Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on Jean-Paul Kress, our Chief Executive Officer, as well as the other principal members of our management and scientific teams. Dr. Kress and such other principal members are employed "at will," meaning we or they may terminate the employment at any time. We do not maintain "key person" insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our product candidates toward scaling up for commercialization, manufacturing and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating

our research and development and commercialization strategy. Our consultants and advisors, including our scientific founder, may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. The inability to recruit, or loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations and prospects.

We expect to expand our development, regulatory and future sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

In connection with the growth and advancement of our pipeline, we expect to increase the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, manufacturing and, as our product candidates advance through later stages of clinical development, sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expected expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

As a growing biotechnology company, we are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for and fully understanding the regulatory and manufacturing pathways to all of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize our product candidates, if approved, will depend in part on our ability to effectively manage the future development and expansion of our company.

Our insurance policies may be inadequate and potentially expose us to unrecoverable risks.

We have limited director and officer insurance and commercial insurance policies. Any significant insurance claims would have a material adverse effect on our business, financial condition and results of operations. Insurance availability, coverage terms and pricing continue to vary with market conditions. We endeavor to obtain appropriate insurance coverage for insurable risks that we identify; however, we may fail to correctly anticipate or quantify insurable risks, we may not be able to obtain appropriate insurance coverage, and insurers may not respond as we intend to cover insurable events that may occur. We have observed rapidly changing conditions in the insurance markets relating to nearly all areas of traditional corporate insurance. Such conditions have resulted in higher premium costs, higher policy deductibles and lower coverage limits. For some risks, we may not have or maintain insurance coverage because of cost or availability.

If our information technology systems, or those of our third-party vendors, collaborators, or other contractors or consultants or other third parties with whom we work, or our data are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to a material disruption of our product development programs, regulatory investigations or actions, litigation, fines and penalties, reputational harm and other adverse consequences.

In the ordinary course of our business, we and the third parties with whom we work process sensitive data, and, as a result, we and such third parties face a variety of evolving threats that could cause security incidents. Our

information technology systems and those of our current and any future third-party vendors, collaborators, consultants or other third parties with whom we work are subject to damage or interruption from a variety of evolving threats, including but not limited to social-engineering attacks (including through deep fakes, which are increasingly more difficult to identify as fake, and phishing attacks), computer viruses, computer hackers, malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), employee theft or misuse, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, denial-of-service attacks, credential stuffing, credential harvesting, ransomware attacks, adware, attacks enhanced or facilitated by AI, sophisticated nation-state and nation-state-supported actors, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive 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.

It may be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks.

Remote work 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 at home, while in transit and in public locations. Additionally, 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.

We rely on third parties to operate critical business systems to process sensitive information in a variety of contexts. 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 the third parties' with whom we work experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if the third parties with whom we work 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. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or that of the third parties with whom we work have not been compromised.

While we seek to protect our information technology systems from system failure, accident and security breach, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other sensitive data or other disruptions. For example, the loss of clinical trial data from completed, ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we were to experience a significant cybersecurity breach of our information systems or sensitive data, the costs associated with the investigation, remediation and potential notification of the breach to counterparties and data subjects could be material. In addition, our remediation efforts may not be successful. If we are unable to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, or the loss of or damage to sensitive data.

Although we have implemented security measures designed to help protect sensitive data from unauthorized access or disclosure, our information technology and infrastructure may be vulnerable in the future to attacks by hackers or viruses, failures, or breaches due to third-party action, employee negligence or error, malfeasance, or

other incidents or disruptions. For example, we have been the target of phishing attacks in the past and we expect such attacks will continue in the future. Furthermore, while we have implemented data privacy and security measures that are designed to comply with applicable laws and regulations relating to privacy and data protection, some health-related and other personal information or confidential information may be transmitted to us or processed by third parties, who may not implement adequate security and privacy measures, and it is possible that laws, rules and regulations relating to privacy, data protection, or information security may be interpreted and applied in a manner that is inconsistent with our practices or those of third parties who transmit health-related and other personal information or confidential information to us or process such information on our behalf. 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.

We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties with whom we work). We may not, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we may experience delays in deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident. Any of the previously identified or similar threats may in the future cause a security incident or other interruption that may in the future result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties with whom we work. A security incident or other interruption could disrupt our ability (and that of third parties) with whom we work to provide our services. We may in the future expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Additionally, certain data privacy and security obligations may require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive data.

To the extent that we or third parties with whom we work are found to have violated data security laws, rules or regulations or if we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, including an incident that results in a loss of, or damage to, our or our third-party vendors', collaborators', consultants' or other third parties with whom we work data or systems, or inappropriate disclosure of confidential or proprietary information, we could experience material adverse consequences including but not limited to litigation exposure (including class claims), government enforcement actions (for example, investigations, penalties and fines, audits, and inspections); additional reporting requirements and/or oversight; indemnification obligations; restrictions on processing sensitive data (including clinical trial data); reputational harm; monetary fund diversions; diversion of management attention; our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects.

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. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive data 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.

Risks Related to the Ownership of Our Common Stock

An active trading market for our common stock may not be sustained.

There is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares. An inactive market may also impair our ability to raise capital to continue to fund operations by selling shares of our common stock and may impair our ability to acquire other companies or technologies by using our common stock as consideration.

The market price of our common stock may be volatile.

Our stock price is, and is likely to continue to be, volatile. For example, our stock traded within a range of a high price of $53.40 and a low price of $3.03 per share for the period of January 1, 2025 through December 31, 2025. As a result of volatility, our stockholders may not be able to sell their common stock at or above the prices at which they purchased their shares. Some of the factors that may cause the market price of our common stock to fluctuate include:

- the success of existing or new competitive product candidates or technologies;
- the timing and results of clinical trials for our product candidates;
- announcements by RemeGen of clinical trial results for telitacicept in RemeGen's development programs in Greater China;
- failure or discontinuation of any of our product development and research programs;
- results of preclinical studies, clinical trials or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- commencement or termination of collaborations for our product development and research programs;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our research programs, product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- global or regional public health emergencies, and geopolitical instability, including terrorist attacks, civil unrest and actual or threatened armed conflict;
- general economic, industry and market conditions, including heightened interest rates and inflation; and
- the other factors described in this "Risk Factors" section.

In recent years, the stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. Following periods of such volatility in the market price of a company's securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future, which could result in substantial costs and divert management's attention and resources from our business.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will be influenced by the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

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

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. Our outstanding shares of common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended (the "Securities Act"), or to the extent such shares have already been registered under the Securities Act and are held by non-affiliates.

Moreover, holders of a substantial number of shares of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. For example, in December 2022 we filed a registration statement on Form S-3 to register the resale of up to 581,395 shares of common stock held by RA Capital Healthcare Fund L.P. which were purchased from us in a private placement, and in January 2025 we filed a registration statement on Form S-3 to register the resale of up to an aggregate of 6,285,515 shares of common stock held by Reprogrammed Interchange LLC and entities affiliated with RA Capital Management, L.P., consisting of (i) 2,793,562 shares of common stock and (ii) 3,491,953 shares of common stock issuable upon the exercise of outstanding warrants to purchase shares of common stock, which were purchased from us in a private placement. We have also filed a registration statement on Form S-3 to register the resale of up to 34,999,999 shares of common stock issuable upon exercise of outstanding warrants, which were purchased from us by certain institutional investors in a private placement, and 16,000,000 shares of common stock that are issuable upon exercise of a warrant to purchase common stock issued to RemeGen as partial consideration for our license agreement with RemeGen, as well as a registration statement on Form S-3 to register the resale of up to 13,876,032 shares of common stock purchased from us in December 2025. We have also agreed to file a registration statement on Form S-3 to register for resale up to 5,338,078 shares of common stock purchased from us in our 2026 Private Placement.

We have also registered or will register all shares of common stock that we may issue under our equity compensation plans or that are issuable upon exercise of outstanding options on a registration statement on Form S-8 and will continue to register any additional shares that become available under such plans due to any annual, automatic increases under the terms of those plans. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

Insiders have substantial control over our company, which could limit the ability of our other stockholders to affect the outcome of key transactions, including a change of control.

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding common stock, and their affiliates, in the aggregate, beneficially own shares representing a substantial amount of our outstanding common stock, as well as warrants to purchase shares of our common stock warrants, which, if exercised, would result in such stockholders owning an even larger percentage of our outstanding common stock. As a result, these stockholders, if they act together, may be able to influence our management and affairs and would control all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions, such as a merger or other sale of our company or its assets. This concentration of ownership may have the effect of delaying or preventing a change in control of our company or discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control, even if that change in

control would benefit our other stockholders. This significant concentration of ownership may also adversely affect the trading price for our common stock because investors often perceive disadvantages in owning stock in companies with controlling stockholders.

If we fail to establish and maintain proper and effective internal control over financial reporting, our operating results and our ability to operate our business could be harmed.

Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be re-evaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles.

Implementing any appropriate changes to our internal controls may distract our officers and employees, entail substantial costs to modify our existing processes and take significant time to complete. These changes may not, however, be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, could increase our operating costs and harm our business. In addition, investors' perceptions that our internal controls are inadequate or that we are unable to produce accurate financial statements on a timely basis may harm our common share price.

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

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). We will be an emerging growth company during this year and may remain an emerging growth company through 2026. 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 other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 ("SOX"), not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved, and being permitted to provide only two years of audited financial statements. As a result, the information we provide stockholders will be different than the information that is available with respect to other public companies. For example, we did not include all of the executive compensation related information in our Annual Report that would be required if we were not an emerging growth company. 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 our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have availed ourselves of this extended transition period and we cannot predict whether investors will find our common stock less attractive due to this election.

We are also a "smaller reporting company" and we may continue to be a smaller reporting company if either (i) the market value of our stock held by non-affiliates is less than $250 million or (ii) our annual revenue is less than $100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging

growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

We 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 are no longer an "emerging growth company," we will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to continue to hire additional accounting, finance and other personnel in connection with our efforts to comply with the requirements of being, a public company, and our management and other personnel will need to continue to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements have increased and will continue to increase our legal and financial compliance costs and make some activities more time-consuming and costly. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, 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.

Pursuant to SOX Section 404, we are required to furnish a report by our management on our internal control over financial reporting, but while we remain an emerging growth or a smaller reporting company with less than $100 million in annual revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To maintain compliance with SOX Section 404 and achieve compliance within the prescribed period for the attestation report by our independent registered public accounting firm, we have and will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants, adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. 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 SOX 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.

Our management team has broad discretion in the use of our cash reserves and may not use them effectively.

Our management has broad discretion to use our cash reserves and could use our cash reserves in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could harm our business, financial condition, results of operations and prospects. Pending their use, we may invest our cash reserves in a manner that does not produce income or that loses value.

We do not expect to pay any dividends for the foreseeable future. Accordingly our stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared or paid any cash dividends on our equity securities. We do not anticipate that we will pay any dividends to holders of our common stock in the foreseeable future. Instead, we plan to retain any earnings to maintain and expand our existing operations. In addition, any future credit facility that we enter into may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

Unfavorable global economic conditions, new tariffs or bank closures could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets, including as a result of heightened inflation and interest rates. A severe or prolonged economic downturn, or additional global financial crises, including related to potential future pandemics, geopolitical issues, armed conflicts or U.S.-China trade and political tensions, could result in a variety of risks to our business, including weakened demand for our product candidates, if approved, or our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

Significant political, trade, or regulatory developments in the United States or other countries in which we have clinical trials, third party suppliers or service providers may have a material adverse effect on us. Similarly, changes in U.S. federal policy that affect the geopolitical landscape could give rise to circumstances outside our control that could have negative impacts on our business operations. We could also be affected by new and increased tariffs between the United States and other countries, including China. These additional tariffs and any retaliatory tariffs by other countries could substantially increase our costs associated with the manufacture and supply of our product candidates. The global trade environment is rapidly evolving, and the United States and other countries may impose additional new tariffs, the scope of which we are unable to predict but that may adversely impact our business. If our or the activities of our third party suppliers or service providers fall within the scope of any of these or other tariffs, our costs may increase significantly. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets. Any changes in political, trade, regulatory, and economic conditions, including U.S. trade policies, could have a material adverse effect on our financial condition or results of operations.

In addition, our available cash and cash equivalents are held in accounts managed by third party financial institutions and consist of cash in our operating accounts and cash invested in money market funds. At any point in time, the funds in our operating accounts may exceed the Federal Deposit Insurance Corporation insurance limits. While we monitor the cash balances in our operating accounts and adjust the cash balances as appropriate, these cash balances could be impacted if the underlying financial institutions fail. We can provide no assurances that access to our operating cash or invested cash and cash equivalents will not be impacted by adverse conditions in the financial markets.

Provisions in our certificate of incorporation and bylaws and under Delaware law could make a change in control of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation and our amended and restated bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;

- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66⅔% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law (the "DGCL"), which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. We have not elected to opt out of DGCL Section 203. These provisions could discourage potential acquisition proposals and could delay or prevent a change in control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in our stockholders' best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our common stock.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising under the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage lawsuits against

us and our directors, officers and other employees. If a court were to find either exclusive-forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

Item 1B. Unresolved Staff Comments.

Not applicable.

Item 1C. Cybersecurity

Risk management and strategy

We have implemented and maintain various information security processes designed to identify, assess and manage material risks from cybersecurity threats to our critical computer networks, third party hosted services, communications systems, hardware and software, and our critical data, including intellectual property, confidential information that is proprietary, and strategic or competitive in nature, and our clinical trial and related data ("Information Systems and Data").

Our information security function, legal, and third-party service providers (collectively, the "Information Security Function") help identify, assess, and manage the Company's cybersecurity threats and risks. The Information Security Function identifies and assesses risks from cybersecurity threats by monitoring and evaluating the Company's threat environment and risk profile using various methods and tools including, for example: manual and automated tools, subscribing to reports and services that identify cybersecurity threats, and evaluating threats reported to us. We have also initiated a third party threat assessment and are implementing ongoing monitoring and response services.

Depending on the environment, systems and data at issue, the Company implements and maintains technical, physical, and organizational measures designed to manage and mitigate material risks from cybersecurity threats to our Information Systems and Data. These measures include, for example: incident detection and response, encryption of certain data, access and network security controls, data segregation, physical security, systems monitoring, employee training, and cybersecurity insurance. Our assessment and management of material risks from cybersecurity threats are integrated into the Company's overall risk management processes. This information is reviewed with the Company's extended leadership team and communicated to the audit committee of the board of directors, which evaluates the Company's risks relating to data privacy, technology and information security, including cybersecurity.

Our assessment and management of material risks from cybersecurity threats are integrated into the Company's overall risk management processes. Key cybersecurity risks are reviewed with the Company's extended leadership team and communicated to the Audit Committee of the Board of Directors, which evaluates the Company's risks relating to data privacy, technology and information security, including cybersecurity.

We leverage third-party service providers to assist us from time to time to identify, assess, and manage material risks from cybersecurity threats, including cybersecurity consultants, cybersecurity software providers, managed cybersecurity service providers, external legal counsel, and dark web monitoring services. We maintain access to third-party forensic investigation services and may engage such services in connection with significant cybersecurity incidents. We also use third-party service providers to perform a variety of functions throughout our business, such as application providers and hosting companies. Depending on the nature of the services provided, the sensitivity of the Information Systems and Data at issue, and the identity of the provider, our vendor management process may involve different levels of assessment designed to help identify cybersecurity risks associated with a provider and impose contractual obligations related to cybersecurity on the provider.

For a description of the risks from cybersecurity threats that may materially affect the Company and how they may do so, see our risk factors under Part 1. Item 1A. Risk Factors in this Annual Report on Form 10-K, including "If our information technology systems, or those of our third-party vendors, collaborators, or other contractors or consultants or other third parties with whom we work, or our data are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to a material disruption of our product development programs, regulatory investigations or actions, litigation, fines and penalties, reputational harm and other adverse consequences."

Governance

Our Board of Directors addresses the Company's cybersecurity risk management as part of its general oversight function. The Board of Directors' Audit Committee is responsible for overseeing Company's cybersecurity risk management processes, including oversight of mitigation of risks from cybersecurity threats.

Our cybersecurity risk assessment and management processes are implemented and maintained by certain Company management, including the Head of Digital, Data and Technology, who oversees the Information Security Management System (ISMS) and Information Security Team. Our Chief Financial Officer is responsible for strategic leadership of our cybersecurity risk management program, hiring appropriate personnel, approving budgets, helping to integrate cybersecurity risk considerations into the Company's overall risk management strategy, and communicating key priorities to relevant personnel. The Head of Digital role is currently held by an individual who has several years of professional IT management experience. He leads the operational and ISMS oversight of company-wide cybersecurity strategy, policy, standards and processes and works across relevant departments to assess and help prepare employees and third-party service providers to address cybersecurity risks. We also have a Director of IT, who oversees our Infrastructure and Service Desk Department.

Our cybersecurity incident response plan is designed to escalate certain cybersecurity incidents to members of management depending on the circumstances, including the General Counsel, the Chief Financial Officer, and the Chief Executive Officer. The Company's information technology department and managed service partners work with the Company's incident response team to help the Company mitigate and remediate cybersecurity incidents of which they are notified. In addition, the Company's incident response plan includes reporting to the Audit Committee of the Board of Directors and the chair of the Board of Directors for certain cybersecurity incidents. The Audit Committee of the Board of Directors periodically receives summaries or presentations from management concerning the Company's significant cybersecurity threats and risk and the processes the Company has implemented to address them.

Item 2. Properties.

Our principal executive office is located at 500 Boylston Street, Suite 1350, Boston, Massachusetts where we lease approximately 8,391 square feet of office space pursuant to a lease that expires in August 2031. We believe that this facility will be adequate for our near-term needs. If required, we believe that suitable additional or alternative space would be available in the future on commercially reasonable terms.

Item 3. Legal Proceedings.

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently subject to any material legal proceedings. Regardless of outcome, such proceedings or claims can have an adverse impact on us because of defense and settlement costs, diversion of resources and other factors, and there can be no assurances that favorable outcomes will be obtained.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

Our common stock trades on the Nasdaq Global Select Market under the symbol "VOR". Trading of our common stock commenced on February 5, 2021 in connection with our initial public offering ("IPO"). Prior to that time, there was no established public trading market for our common stock.

Holders

As of March 23, 2026, we had approximately 77 holders of record of our common stock. This number does not include beneficial owners whose shares were held in street name.

Dividend Policy

We have never declared or paid any cash dividends on our common stock. We currently intend to retain future earnings to fund the development and growth of our business. We do not expect to pay any cash dividends in the foreseeable future. In addition, the terms of any future debt agreements that we may enter into, may preclude us from paying dividends without the lenders' consent or at all.

Item 6. [Reserved]

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

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K (the "Annual Report"). Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the section titled "Risk Factors," our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

Vor Bio is a clinical-stage biopharmaceutical company focused on developing a novel therapy in the treatment of autoimmune diseases. In June 2025, we in-licensed telitacicept from RemeGen Co., Ltd. ("RemeGen"). Pursuant to our license agreement with RemeGen, we were granted an exclusive license to develop and commercialize telitacicept outside of the Greater China region, which includes mainland China, Hong Kong, Macau and Taiwan. RemeGen retains development and commercialization rights in Greater China. Telitacicept is approved in China for the treatment of generalized myasthenia gravis ("gMG"), systemic lupus erythematosus ("SLE") and rheumatoid arthritis ("RA"), and has two Biologics License Applications ("BLAs") filed and pending in China for the treatment of Sjögren's disease ("SjD") and IgA nephropathy ("IgAN").

Telitacicept is currently being evaluated in a global Phase 3 clinical trial, for which we have assumed responsibility from RemeGen in connection with the license agreement, for the treatment of gMG. The trial is currently recruiting patients in North America, Europe, Latin America, and Asia to support potential approval in the United States, Europe, Japan and other countries. In July 2024, the clinical trial enrolled a patient in the United States, the first in the global clinical trial. Topline data from the trial is anticipated in the first half of 2027.

Telitacicept was evaluated by RemeGen in a Phase 3 clinical trial in patients with gMG in China. Most recently, the 48-week data from Part B of the Phase 3 trial were presented at the American Association of Neuromuscular & Electrodiagnostic Medicine ("AANEM") Annual Meeting in October 2025.

We have recently initiated a global Phase 3 clinical trial evaluating telitacicept for the treatment of SjD, with first patient dosing in March 2026. The trial anticipates recruiting approximately 250 adults with SjD in the United States, Europe, South America, and Asia. The trial is a randomized, double-blind, placebo-controlled trial.

Telitacicept was evaluated by RemeGen in a Phase 3 clinical trial in patients with active SjD in China. Most recently, the 48-week data including Stage A and B from the Phase 3 trial were presented at the American College of Rheumatology ("ACR") Annual Meeting in October 2025.

We have incurred significant operating losses since inception, including net losses of $696.0 million for the year ended December 31, 2025 and $116.9 million for the year ended December 31, 2024. As of December 31, 2025, we had an accumulated deficit of $1,153.0 million.

As of December 31, 2025, we had cash, cash equivalents and marketable securities of $455.2 million. Based on our current operating plan, we expect that our cash, cash equivalents and marketable securities, together with the expected proceeds from our 2026 Private Placement, will enable us to fund our operating expenses and capital expenditure requirements into early 2029.

Restructuring Plan

On May 5, 2025, our board of directors approved the wind down of our then-existing clinical and manufacturing operations focused on previous product candidates (the "Restructuring Plan"). We publicly announced this plan on May 8, 2025. In conjunction with the Restructuring Plan, we announced a reduction of our workforce by 154 full-time employees, or approximately 99% of our then-current employee base.

During the year ended December 31, 2025, we incurred restructuring costs related to the Restructuring Plan of $29.7 million comprised of severance payments, stock-based compensation modifications, loss on disposal of long-lived assets and accelerated depreciation and amortization on long-lived assets and right-of-use assets.

Financial Operations Overview

Revenue

We have not generated any revenue since our inception and do not expect to generate any revenue from the sale of products in the near future, if at all. If our development efforts for our product candidates are successful and result in marketing approval, or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from a combination of product sales or payments from such agreements.

Expenses

Research and Development Expenses

Research and development expenses consist primarily of external and internal expenses incurred in connection with our research and development activities, including our drug discovery efforts and the development of our product candidates. External expenses include:

- research and development expenses incurred under agreements with clinical research organizations ("CROs") and other scientific development services;
- costs of consultants, including their fees and related travel expenses;
- costs related to compliance with quality and regulatory requirements;
- costs of laboratory supplies and acquiring and developing preclinical and clinical trial materials, including expenses associated with our clinical manufacturing organizations ("CMOs"); and
- payments made and consideration issued under third party licensing agreements.

Internal expenses include:

- personnel-related expenses, including salaries, bonuses, benefits and stock-based compensation expenses, for employees involved in research and development activities;
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent, insurance, and other internal operating costs; and
- research and development related restructuring costs incurred with the Restructuring Plan, including severance payments, stock-based compensation modifications, loss on disposal of long-lived assets and accelerated depreciation and amortization on long-lived assets and right-of-use assets.

We expense 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 consolidated financial statements as prepaid expenses or accrued research and development expenses. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized, even when there is no alternative future use for the research and development. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

A significant portion of our research and development costs have been external costs, which we track by program.

Research and development activities are central to our business model. We expect that our research and development expenses will increase significantly for the foreseeable future as we continue to identify and develop product candidates, particularly as our product candidates move into later stages of clinical development.

The successful development of our product candidates in the future is highly uncertain. Therefore, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development and commercialization of any of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from the sale of our product candidates, if approved. This is due to the numerous risks and uncertainties associated with developing product candidates, many of which are outside of our control, including the uncertainty of:

- the timing and progress of clinical development activities;
- the number and scope of clinical programs we decide to pursue;
- our ability to maintain our current research and development programs and to establish new ones;
- establishing an appropriate safety profile with IND-enabling studies;
- the number of sites and patients included in the clinical trials;
- the countries in which the clinical trials are conducted;
- per patient trial costs;
- successful patient enrollment in, and the initiation of, clinical trials, as well as drop out or discontinuation rates;
- the successful completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the number of trials required for regulatory approval;
- the timing, receipt and terms of any regulatory approvals from applicable regulatory authorities;
- our ability to establish new licensing or collaboration arrangements;
- the performance of our current and future collaborators, if any;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- significant and changing government regulation and regulatory guidance;
- the impact of any business interruptions to our operations or to those of the third parties with whom we work;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- launching commercial sales of our product candidates, if approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of the product candidates following approval.

Any changes in the outcome of any of these variables could mean a significant change in the costs and timing associated with the development of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel-related costs, including salaries, bonuses, benefits and stock-based compensation expenses for employees involved in our executive, finance, corporate, business development and administrative functions, as well as expenses for outside professional services, including legal, audit, accounting and tax-related services and other consulting fees, facility-related expenses, which include depreciation costs and other allocated expenses for rent and maintenance of facilities, insurance costs, recruiting costs, travel expenses and other general administrative expenses. General and administrative costs also consist of restructuring costs incurred under the Restructuring Plan, including severance payments, stock-based compensation modifications, and accelerated depreciation and amortization on long-lived assets and right-of-use assets.

We expect that our general and administrative expenses will increase as our business expands and we hire additional personnel to support our continued development of our clinical programs. We also anticipate continued increased expenses associated with being a public company, including costs for legal, audit, accounting, investor and public relations, regulatory and tax-related services related to compliance with the rules and regulations of the SEC, Nasdaq listing standards and director and officer insurance premiums.

Other Income (Expense)

Interest Income

Interest income consists of interest income earned on our cash, cash equivalents and marketable securities held in financial institutions.

Other Income

Other income represents the proceeds received from the sale of certain intellectual property related to our previous product candidates trem-cel, VCAR33 and VADC45.

Change in Fair Value of Warrant Liabilities

Change in fair value of warrant liabilities represents the change in the fair value of liability-classified warrants due to changes in their intrinsic value resulting from changes in the quoted price of our common stock underlying the warrants.

Results of Operations

Comparison of Years Ended December 31, 2025 and 2024

The following table summarizes our results of operations for the years ended December 31, 2025 and 2024 (in thousands):

	Year Ended December 31,		
	2025	2024	Change
Operating expenses:			
Research and development	$ 321,532	$ 93,310	$ 228,222
General and administrative	50,110	27,875	22,235
Total operating expenses	371,642	121,185	250,457
Loss from operations	(371,642)	(121,185)	(250,457)
Other income:			
Interest income	5,868	4,271	1,597
Other income	4,151	—	4,151
Change in fair value of warrant liabilities	(334,358)	—	(334,358)
Total other (expense) income	(324,339)	4,271	(328,610)
Net loss	$ (695,981)	$ (116,914)	$ (579,067)

Research and Development Expenses

The following table summarizes our research and development expenses incurred for the years ended December 31, 2025 and 2024 (in thousands):

	Year Ended December 31,		
	2025	2024	Change
External research expenses:			
Telitacicept - gMG	$ 24,959	—	$ 24,959
Telitacicept - SjD	3,300	—	3,300
Trem-cel	9,437	18,905	(9,468)
VCAR33	6,000	8,930	(2,930)
Other research and development	229,092	12,608	216,484
Internal research expenses:			
Salaries and benefits (including stock-based compensation)	26,389	38,309	(11,920)
Manufacturing, facilities, and other research expenses	22,355	14,558	7,797
Total research and development expenses	$ 321,532	$ 93,310	$ 228,222

Research and development expenses were $321.5 million for the year ended December 31, 2025, compared to $93.3 million for the year ended December 31, 2024. The increase of $228.2 million was primarily attributable to the $222.6 million of expense incurred in 2025 for the purchase of the telitacicept license (included as a component of other research and development), a $25.0 million increase due to new spend for Telitacicept-gMG, an increase in manufacturing, facilities, and other expenses of $7.8 million primarily due to lease impairments taken in 2025 in connection with the Restructuring Plan, and a $3.3 million increase due to new spend for Telitaciept- SjD. These increases were offset in part by a $11.9 million decrease in personnel-related costs due to the Restructuring Plan, and decreases of $9.5 million in Trem-cel spend and $2.9 million in VCAR33 spend due to the discontinuation of those programs in the first half of 2025. In addition, the increase in Other research and development attributed to the telitacicept license was partially offset by a decrease of $7.1 million in non-license spend, attributable primarily to decreases in lab supplies and consumables, consulting fees, and software expenses, due to our decrease in related activities as part of the Restructuring Plan.

General and Administrative Expenses

General and administrative expenses were $50.1 million for the year ended December 31, 2025, compared to $27.9 million for the year ended December 31, 2024. The increase of $22.2 million was primarily attributable to an increase of $11.8 million in stock-based compensation, an increase of $4.9 million in legal and professional fees, an increase of $3.9 million in personnel costs, and an increase of $1.6 million in facilities, equipment, and other costs. The increase in stock-based compensation was primarily driven by grants to new hires, including grants to the new executives hired during the year, as well as an appreciation in our stock price and incremental expense recognized from award modifications which took place during the year. The increase in personnel costs was driven by the severance costs incurred in connection with the Restructuring Plan, partially offset by a reduction in headcount compared to the prior year. The increase in legal and professional fees was driven primarily by increased consulting costs incurred in the current year compared to prior year due to our transition after the implementation of the Restructuring Plan. The increase in these fees was also attributable to an increase in legal fees driven by the increase in transactions which occurred during the year, as well as accounting fees relating to incremental reviews of significant transactions. The increase in facilities and other costs was primarily driven by an increase in rent expense allocated to general and administrative expense as a result of the shift in headcount brought on by the Restructuring Plan, and an increase in software related expenses in the current year.

Other Income (Expense), net

Other income (expense), net decreased by $328.6 million for the year ended December 31, 2025, compared to the year ended December 31, 2024. The decrease was primarily due to the $334.4 million loss on the change in fair value of warrant liabilities, partially offset by a $1.6 million increase in interest income due to an increase in cash, cash equivalents and marketable securities and $4.1 million of income recognized from the sale of intellectual property in 2025.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have not recognized any revenue and have incurred operating losses and negative cash flows from our operations. We have not yet commercialized any product and we do not expect to generate revenue from sales of any products for several years, if at all. We have funded our operations primarily through the sale of equity securities and have received aggregate net proceeds from these transactions of approximately $1,019.1 million as of December 31, 2025.

In order to fund our future operations, including our ongoing and planned clinical trials, we filed a universal shelf registration statement, which was declared effective on March 31, 2025, to provide for aggregate offerings of up to $350.0 million of common stock, preferred stock, debt securities, warrants or any combination thereof. As of December 31, 2025, $164.2 million remained available under the shelf registration statement, including $48.9 million reserved for at-the market offerings discussed below.

At-the-Market Sales Agreements

In December 2022, we entered into a Sales Agreement with Stifel, Nicolaus & Company, Incorporated ("Stifel") as the agent (the "Stifel ATM Facility"). Pursuant to the Stifel ATM Facility, we may offer and sell shares of common stock with an aggregate value of up to $125.0 million. We pay Stifel a commission of up to 3.0% of the gross proceeds of any common stock sold through Stifel. We sold 1,910,861 and 12,454 shares of common stock under the Stifel ATM Facility during the years ended December 31, 2025 and 2024, respectively at a weighted average price per share of $37.06 and $32.09, respectively, for aggregate net proceeds of $70.1 million and $0.3 million, respectively, after deducting commissions. As of December 31, 2025, $48.9 million remained available to be sold under the Stifel ATM Facility.

2024 Private Placement

On December 27, 2024, we entered into a purchase agreement with certain institutional investors pursuant to which we issued and sold in a private placement an aggregate of (i) 2,793,562 shares of common stock and (ii) warrants to purchase up to 3,491,953 shares of common stock at the closing of the private placement on December 30, 2024 (the "December 2024 Private Placement"). Net proceeds from the private placement were $52.7 million, after deducting placement fees and issuance costs payable by us. If exercised for cash, the warrants would result in additional gross proceeds to us of up to approximately $58.5 million.

June 2025 Private Placement

On June 25, 2025, we entered into a purchase agreement with certain institutional investors, pursuant to which we issued and sold in a private placement pre-funded warrants to purchase up to an aggregate of 34,999,999 shares of common stock (the "2025 PIPE Warrants") at the closing on June 27, 2025 (the "June 2025 Private Placement"). Net proceeds from the private placement were $174.4 million, after deducting issuance costs payable by us.

November 2025 Public Offering

On November 10, 2025, we entered into an underwriting agreement relating to the issuance and sale in a public offering of 11,500,000 shares of common stock, including 1,500,00 shares purchased by the underwriters under a 30-day option to purchase additional shares (the "November 2025 Offering") at a public offering price of $10.00 per share. The net proceeds from the November 2025 Offering were $107.7 million after deducting the underwriting discounts and commissions and offering expenses.

December 2025 Private Placement

On December 15, 2025, we entered into a purchase agreement with certain investors pursuant to which we issued and sold an aggregate of 13,876,032 shares of common stock, at a price per share of $10.81, for net proceeds of $149.9 million after deducting issuance costs payable by us (the "December 2025 Private Placement").

March 2026 Private Placement

On March 26, 2026, we entered into a purchase agreement with certain institutional investors pursuant to which we agreed to issue and sell an aggregate of 5,338,078 shares of common stock, at a price per share of $14.05, for gross proceeds of $75.0 million before deducting issuance costs payable by us. The closing of the private placement is expected to occur on March 30, 2026, subject to satisfaction of customary closing conditions.

Cash Requirements

As of December 31, 2025, we had cash, cash equivalents and marketable securities of $455.2 million. We will need to raise additional capital to fund our planned future operations.

We expect that our existing cash, cash equivalents and marketable securities at December 31, 2025, together with the expected proceeds from our 2026 Private Placement, will enable us to fund our operating expenses and capital expenditure requirements into early 2029. We have based this estimate on assumptions that may prove to be wrong and we could exhaust our capital resources sooner than we expect.

We expect our expenses to increase substantially if, and as, we:

- continue clinical development of our product candidate and any future product candidates, including in particular the expenses associated with our clinical trials;
- incur third party manufacturing costs to support our clinical trials of our product candidate and any future product candidates and, if approved, their commercialization;
- seek to identify and develop additional product candidates;
- seek regulatory and marketing approvals for our product candidate and any future product candidates;
- establish a sales, marketing and distribution infrastructure to commercialize any approved product candidates;
- adapt our regulatory compliance efforts to incorporate requirements to applicable marketed products;
- acquire or in-license products, product candidates, or technologies;
- maintain, expand, enforce, defend and protect our intellectual property;
- hire additional clinical, quality control, manufacturing and other scientific personnel;
- add operational, financial and management information systems and personnel;
- expand our office facility or establish dedicated laboratory and manufacturing facilities; and
- experience any delays or encounter any issues with any of the above.

In addition, we expect to continue to incur additional costs associated with operating as a public company, including significant legal, audit, accounting, investor and public relations, regulatory, tax-related, director and officer insurance premiums, investor relations and other expenses. Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval for any product candidates or generate revenue from the sale of any product candidate for which we may obtain marketing approval. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for at least several years, if ever.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through the public or private sale of our equity, government or private party grants, debt financings or other capital sources, including potential collaborations with other companies or other strategic transactions. To the extent that we raise additional capital through the sale of our equity or convertible debt

securities, including through the use of the Stifel ATM Facility, the ownership interest of our shareholders 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 are unable to obtain additional funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or any commercialization efforts, which could adversely affect our business prospects, or we may be unable to continue operations. If we raise funds through strategic collaborations or other similar arrangements with third parties, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs or product candidates or may have to grant licenses on terms that may not be favorable to us and/or may reduce the value of our common stock. Our ability to raise additional funds may be adversely impacted by worsening global economic conditions and disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from worsening geopolitical tensions and adverse macroeconomic conditions or otherwise. Because of the numerous risks and uncertainties associated with product development, we cannot predict the timing or amount of increased expenses, and there is no assurance that we will ever be profitable or generate positive cash flow from operating activities.

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements that have, or are reasonably likely to have, a material current or future effect on our financial condition, changes in financial condition, revenues or expenses, results of operations, liquidity, cash requirements or capital resources.

Cash Flows

The following table provides information regarding our cash flows for the periods presented (in thousands):

	Year Ended December 31,	
	2025	**2024**
Net cash used in operating activities	$ (142,713)	$ (99,660)
Net cash (used in) provided by investing activities	(48,802)	96,857
Net cash provided by financing activities	503,807	53,392
Net increase in cash, cash equivalents and restricted cash equivalents	$ 312,292	$ 50,589

Operating Activities

Net cash used in operating activities was $142.7 million for the year ended December 31, 2025, primarily reflecting a net loss of $696.0 million, offset primarily by non-cash charges of $543.1 million. The non-cash charges primarily consisted of the fair value of warrants issued in connection with entering into the Telitacicept License Agreement of $177.4 million, a $334.4 million loss due to the change in fair value of warrant liabilities, stock-based compensation expense of $18.9 million, non-cash lease expense of $5.6 million, a $3.3 million loss on the sale of equipment, and depreciation expense of $2.9 million, offset by non-cash interest accretion of $0.1 million. Changes in working capital balances increased by a net of $10.2 million during the year, driven primarily by an increase in accrued expenses and accounts payable due to increases in accrued clinical and manufacturing expenses, partially offset by a decrease in accrued expenses for personnel costs. The net cash used was also impacted by the $45.0 million payment made to Remegen for the telitacicept license.

Net cash used in operating activities was $99.7 million for the year ended December 31, 2024, primarily reflecting a net loss of $116.9 million, offset primarily by non-cash charges of $17.2 million. The non-cash charges primarily consisted of stock-based compensation expense of $9.8 million, non-cash lease expense of $5.0 million and depreciation expense of $3.5 million, offset by non-cash interest accretion of $1.1 million.

Investing Activities

Net cash used in investing activities was $48.8 million for the year ended December 31, 2025, which consisted of purchases of $53.7 million of marketable securities and $0.9 million of property and equipment, partially offset by proceeds of $5.0 million from the maturity of marketable securities and $0.8 million from the sale of equipment.

Net cash provided by investing activities was $96.9 million for the year ended December 31, 2024, which consisted of purchases of $9.9 million of marketable securities and $0.2 million of property and equipment offset by proceeds of $107.0 million from the maturity of marketable securities.

Financing Activities

Net cash provided by financing activities was $503.8 million for the year ended December 31, 2025, which consisted of $175.0 million of proceeds from the June 2025 Private Placement, $150.0 million of proceeds from the December 2025 Private Placement, $108.1 million of proceeds received from the November 2025 Offering, $70.1 million of proceeds from the sale of common stock under the Stifel ATM Facility, and proceeds of $2.2 million from the exercise of stock options and purchases of common stock under our ESPP. These amounts were offset by the payment of $1.3 million of issuance costs related to the private placements and underwritten offering and $0.3 million of taxes paid related to net share settlement of equity awards.

Net cash provided by financing activities was $53.4 million for the year ended December 31, 2024, which consisted of proceeds of $55.6 million from the proceeds of the December 2024 Private Placement, $0.3 million from the sale of common stock under the Stifel ATM Facility and proceeds of $0.2 million from the exercise of stock options and purchases of common stock under our ESPP, offset by the payment of $2.3 million of issuance costs related to the private placement and $0.3 million of taxes paid related to net share settlement of equity awards.

Contractual Obligations and Other Commitments

Contractual obligations relate to future minimum lease payments for our existing non-cancellable lease relating to corporate office space, with a term expiring in August 2031. Future minimum annual rental payments required under this operating lease agreement as of December 31, 2025 are described in more detail in Note 9 to our audited consolidated financial statements included elsewhere in this Annual Report.

Other commitments include license and collaboration agreements we have entered into with certain parties. Such arrangements require ongoing payments, including payments upon the achievement of certain development, regulatory and commercial milestones, receipt of sublicense income, as well as royalties on commercial sales. Refer to Note 10 to our audited consolidated financial statements included elsewhere in this Annual Report.

We also have agreements with certain vendors for various services, including services related to clinical operations and support, which we are not contractually able to terminate for convenience and avoid any and all future obligations to the vendors. Under such agreements, we are contractually obligated to make certain payments to vendors to reimburse them for their unrecoverable outlays incurred prior to cancellation. The exact amounts of such obligations are dependent on the timing of termination and the exact terms of the relevant agreement and cannot be reasonably estimated. We do not include these payments in this summary as they are not fixed and estimable.

Critical Accounting Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our consolidated financial statements. Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and

assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions. During the year ending December 31, 2025, there were no material changes to these assumptions.

While our significant accounting policies are described in more detail in Note 2 to our audited consolidated financial statements included elsewhere in this Annual Report, we believe that the following accounting policy is the most critical in the preparation of our consolidated financial statements.

Accrued Research and Development Expenses

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

Recent Accounting Pronouncements

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

Emerging Growth Company and Smaller Reporting Company Status

Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We elected the extended transition period for complying with new or revised accounting standards, which delays the adoption of these accounting standards until they would apply to private companies.

In addition, as an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- being permitted to present only two years of audited consolidated financial statements in addition to any required unaudited interim consolidated financial statements, with correspondingly reduced disclosure in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations";
- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements;
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements; and
- an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor's report on consolidated financial statements.

We may take advantage of these provisions until the last day of the fiscal year ending after the fifth anniversary of our initial public offering or such earlier time that we no longer qualify as an emerging growth company. We will cease to qualify as an emerging growth company on the date that is the earliest of: (i) December 31, 2026; (ii) the last day of the fiscal year in which we have more than $1.235 billion in total annual gross revenues; (iii) the date on which we are deemed to be a "large accelerated filer" under the rules of the SEC, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June

30th and we have been a public company for at least 12 months and have filed one annual report on Form 10-K; or (iv) the date on which we have issued more than $1.0 billion of non-convertible debt over the prior three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens. We have taken advantage of certain reduced reporting requirements in this this Annual Report. Accordingly, the information contained herein may be different than you might obtain from other public companies in which you hold equity interests.

We are also a "smaller reporting company." If we are a smaller reporting company at the time that we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited consolidated financial statements in our Annual Report and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

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

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

Item 8. Financial Statements and Supplementary Data.

The financial statements required to be filed pursuant to this Item 8 are appended to this Annual Report on Form 10-K. An index of those financial statements is found in Item 15, Exhibits and Financial Statement Schedules, of this Annual Report on Form 10-K.

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

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2025. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934 (the "Exchange Act"), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2025, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Annual Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Rule 13a-15(f) under the Exchange Act. Our management conducted an assessment of the effectiveness of our internal control over financial reporting based on the criteria set forth in "Internal Control-Integrated Framework (2013 framework)" issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on this assessment, management concluded that, as of December 31, 2025 our internal control over financial reporting was effective.

Attestation Report of the Registered Public Accounting Firm

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm on internal control over financial reporting due to an exemption established by the JOBS Act for "emerging growth companies."

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during our most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

During the three months ended December 31, 2025, no director or officer, as defined in Rule 16a-1(f) under the Exchange Act, adopted or terminated a "Rule 10b5-1 trading arrangement" or a "non-Rule 10b5-1 trading arrangement," each as defined in Regulation S-K Item 408.

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

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this Item 10 will be set forth under the captions “Executive Officers”, “Proposal No. 1 – Election of Directors,” “Corporate Governance,” and "Delinquent Section 16(a) Reports", if applicable, in our Definitive Proxy Statement with respect to our 2026 Annual Meeting of Stockholders to be filed with the SEC and is incorporated herein by reference.

Item 11. Executive Compensation.

The information required by this Item 11 will be set forth under the captions “Executive Compensation” and “Director Compensation” in our Definitive Proxy Statement with respect to our 2026 Annual Meeting of Stockholders to be filed with the SEC and is incorporated herein by reference.

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

The information required by this Item 12 will be set forth under the captions “Security Ownership of Certain Beneficial Owners and Management” and “Equity Compensation Plan Information at December 31, 2025” in our Definitive Proxy Statement with respect to our 2026 Annual Meeting of Stockholders to be filed with the SEC and is incorporated by reference.

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

The information required by this Item 13 will be set forth under the captions “Certain Relationships and Related Party Transactions” and “Corporate Governance” in our Definitive Proxy Statement with respect to our 2026 Annual Meeting of Stockholders to be filed with the SEC and is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services.

The information required by this Item 14 will be set forth under the caption “Independent Registered Public Accountants’ Fees” in our Definitive Proxy Statement with respect to our 2026 Annual Meeting of Stockholders to be filed with the SEC and is incorporated herein by reference.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(1) For a list of the financial statements included herein, see Index to the Consolidated Financial Statements on page F-1 of this Annual Report on Form 10-K, incorporated into this Item by reference.

(2) Schedules have been omitted since they are either not required or not applicable or the information is otherwise included herein.

(3) Exhibits

Exhibit Number	Description	Incorporated by Reference				Filed Herewith
		Form	File No.	Exhibit Number	Filing Date	
3.1	Amended and Restated Certificate of Incorporation of the Registrant	8-K	001-39979	3.1	February 9, 2021	
3.2	Certificate of Amendment of Amended and Restated Certificate of Incorporation of the Registrant.	8-K	001-39979	3.1	May 23, 2025	
3.3	Certificate of Amendment of Amended and Restated Certificate of Incorporation of the Registrant.	8-K	001-39979	3.1	September 17, 2025	
3.4	Amended and Restated Bylaws of the Registrant	8-K	001-39979	3.2	February 9, 2021	
4.1	Form of Common Stock Certificate of the Registrant	S-1/A	333-252175	4.1	February 1, 2021	
4.2	Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated June 30, 2020	S-1/A	333-252175	4.2	February 1, 2021	
4.3	Description of the Registrant's Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934					X
4.4	Form of Common Stock Warrant	8-K	001-39979	4.1	December 27, 2024	
4.5	Form of Pre-Funded Warrant to Purchase Common Stock	8-K	001-39979	10.1	June 26, 2025	
10.1†	License Agreement, dated as of June 25, 2025, by and between Remegen Co, Ltd. and the Registrant	10-Q	001-39979	10.9	August 12, 2025	
10.2^	Lease Agreement, dated August 5, 2025, by and between the Company and 500 Boylston & 222 Berkeley Owner (DE) LLC	10-Q	001-39979	10.10	August 12, 2025	
10.3+	2015 Stock Incentive Plan and Forms of Option Grant Agreements, Exercise	S-1	333-252175	10.5	January 15, 2021	

	Notices and Restricted Stock Agreement					
10.4+	2021 Equity Incentive Plan and Forms of Stock Option Grant Notice, Stock Option Agreement, Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement	S-1/A	333-252175	10.6	February 1, 2021	
10.5+	Amended and Restated 2021 Equity Incentive Plan	8-K	001-39979	10.1	August 27, 2025	
10.6+	2021 Employee Stock Purchase Plan	S-1/A	333-252175	10.7	February 1, 2021	
10.7+	2023 Inducement Plan and Forms of Stock Option Grant Notice, Stock Option Agreement, Notice of Exercise, RSU Award Grant Notice and Award Agreement (RSU Award) thereunder	S-8	333-274275	99.1	August 30, 2023	
10.8+	Form of Indemnification Agreement with Executive Officers and Directors	S-1	333-252175	10.8	January 15, 2021	
10.9+	Offer Letter, by and between the Registrant and Robert Ang, dated June 28, 2019	S-1	333-252175	10.9	January 15, 2021	
10.10+	Transition Agreement, dated as of June 25, 2025, by and between the Registrant and Robert Ang	8-K	001-39979	10.6	June 26, 2025	
10.11+	Employment Agreement, dated as of June 25, 2025, by and between the Registrant and Jean-Paul Kress	8-K	001-39979	10.7	June 26, 2025	
10.12+	Employment Agreement, dated as of July 9, 2025, by and between the Registrant and Sandesh Mahatme	8-K	001-39979	10.1	July 10, 2025	
10.13+	Employment Agreement, dated as of July 17, 2025, by and between the Registrant and Qing Zuraw	8-K	001-39979	10.1	July 22, 2025	
10.14+	Amendment to Employment Agreement, dated November 2, 2025, by and between the Registrant and Qing Zuraw	10-Q	001-39979	10.6	November 13, 2025	
10.15+	Non-Employee Director Compensation Policy					X
10.16	Sales Agreement, dated December 23, 2022, by and between the Registrant and Stifel, Nicolaus & Company, Incorporated	8-K	001-39979	1.1	December 23, 2022	
10.17†	Form of Securities Purchase Agreement, dated June 25, 2025, by and between the Registrant and Yantai	8-K	001-39979	10.2	June 26, 2025	

	Rongpu Investment Partnership (Limited Partnership)					
10.18	Form of Securities Purchase Agreement, dated June 25, 2025, by and between the Registrant and the investors named therein	8-K	001-39979	10.3	June 26, 2025	
10.19	Form of Securities Purchase Agreement, dated December 15, 2025, by and between the Registrant and the investors named therein	8-K	001-39979	10.1	December 18, 2025	
10.20	Form of Registration Rights Agreement, dated June 25, 2025, by and between the Registrant and the investors named therein	8-K	001-39979	10.4	June 26, 2025	
10.21	Form of Registration Rights Agreement, dated December 15, 2025, by and between the Registrant and the investors named therein	8-K	001-39979	10.2	December 18, 2025	
10.22	Form of Support Agreement, dated June 25, 2025, by and between the Registrant and the investors named therein	8-K	001-39979	10.5	June 26, 2025	
19.1	Insider Trading and Window Period Policy	10-K	001-39979	19.1	March 20, 2025	
21.1	Subsidiaries of the Registrant	S-1	333-252175	21.1	January 15, 2021	
23.1	Consent of Ernst & Young LLP					X
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002					X
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002					X
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002					X
97.1	Incentive Compensation Recoupment Policy	10-K	001-39979	97.1	March 20, 2025	

101.INS	Inline XBRL Instance Document	X
101.SCH	Inline XBRL Taxonomy Extension Schema Document	X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document	X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document	X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document	X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document	X
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)	X

+ Indicates management contract or compensatory plan.

† Portions of the exhibit have been omitted as the Registrant has determined that: (i) the omitted information is not material; and (ii) the omitted information is the type that the Registrant treats as private or confidential.

^ Schedules and similar attachments have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The Registrant will furnish a supplemental copy of any omitted schedule or similar attachment to the SEC upon request.

***** This certification is being furnished and will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, and are not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

Item 16. Form 10-K Summary

None.

SIGNATURES

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

VOR BIOPHARMA INC.

Date: March 30, 2026

By: /s/ Jean-Paul Kress
Jean-Paul Kress, M.D.
President, Chief Executive Officer and Chairman

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

Name	Title	Date
/s/ Jean-Paul Kress Jean-Paul Kress, M.D.	President, Chief Executive Officer and Chairman (Principal Executive Officer)	March 30, 2026
/s/ Sandesh Mahatme Sandesh Mahatme	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 30, 2026
/s/ Daniella Beckman Daniella Beckman	Lead Independent Director	March 30, 2026
/s/ Alexander Cumbo Alexander Cumbo	Director	March 30, 2026
/s/ Erez Kalir Erez Kalir	Director	March 30, 2026
/s/ Michel Detheux Michel Detheux, Ph.D.	Director	March 30, 2026
/s/ Wouter Joustra Wouter Joustra	Director	March 30, 2026
/s/ Fouad Namouni Fouad Namouni, M.D.	Director	March 30, 2026
/s/ Andrew Levin Andrew Levin, M.D., Ph.D.	Director	March 30, 2026

VOR BIOPHARMA INC.
INDEX TO FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Stockholders and the Board of Directors of Vor Biopharma Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Vor Biopharma Inc. (the Company) as of December 31, 2025 and 2024, the related consolidated statements of operations and comprehensive loss, stockholders' equity (deficit) and cash flows for each of the two years in the period ended December 31, 2025, 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, 2025 and 2024, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2025, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the 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 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 financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

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

Boston, Massachusetts

March 30, 2026

VOR BIOPHARMA INC.
CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share amounts)	December 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 396,486	$ 81,949
Marketable securities	58,722	9,977
Prepaid expenses	1,152	4,201
Other current assets	1,745	380
Total current assets	458,105	96,507
Restricted cash equivalents	168	2,413
Property and equipment, net	533	6,581
Operating lease right-of-use assets	2,936	35,007
Other assets	2,384	2,383
Total assets	$ 464,126	$ 142,891
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 5,127	$ 1,505
Accrued liabilities	19,764	12,892
Operating lease liabilities	280	4,215
Total current liabilities	25,171	18,612
Non-current liabilities:		
Operating lease liabilities—non-current	2,720	27,615
Warrant liabilities	600,547	—
Total liabilities	628,438	46,227
Stockholders' equity (deficit):		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized as of December 31, 2025 and December 31, 2024; 0 shares issued and outstanding as of December 31, 2025 and December 31, 2024	—	—
Common stock, $0.0001 par value; 800,000,000 and 400,000,000 shares authorized as of December 31, 2025 and December 31, 2024, respectively; 38,720,196 and 6,238,799 shares issued and outstanding as of December 31, 2025 and December 31, 2024, respectively	18	13
Additional paid-in capital	988,604	553,623
Accumulated other comprehensive income	41	22
Accumulated deficit	(1,152,975)	(456,994)
Total stockholders' equity (deficit)	(164,312)	96,664
Total liabilities and stockholders' equity (deficit)	$ 464,126	$ 142,891

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

VOR BIOPHARMA INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except share and per share amounts)	Year Ended December 31, 2025	2024
Operating expenses:		
Research and development	$ 321,532	$ 93,310
General and administrative	50,110	27,875
Total operating expenses	$ 371,642	$ 121,185
Loss from operations	$ (371,642)	$ (121,185)
Other income (expense), net:		
Interest income	5,868	4,271
Other income	4,151	—
Change in fair value of warrant liabilities	(334,358)	—
Total other income (expense), net	(324,339)	4,271
Net loss	$ (695,981)	$ (116,914)
Net loss per share, basic and diluted	$ (70.50)	$ (34.03)
Weighted-average common shares outstanding, basic and diluted	9,871,866	3,435,533
Other comprehensive income:		
Unrealized gain on available for sale marketable securities	19	99
Total other comprehensive income	19	99
Comprehensive loss	$ (695,962)	$ (116,815)

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

VOR BIOPHARMA INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (DEFICIT)

(in thousands, except share amounts)	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
Balance, December 31, 2023	3,395,072	$ 7	$ 490,874	$ (77)	$ (340,080)	$ 150,724
Issuance of common stock upon vesting of RSUs, net of shares withheld for taxes, exercise of stock options, and issuance of common stock under ESPP	37,711	—	(56)	—	—	(56)
Issuance of common stock from at-the-market sales agreement	12,454	—	302	—	—	302
Stock-based compensation expense	—	—	9,847	—	—	9,847
Issuance of common stock from private placement, net of issuance costs	2,793,562	6	52,656			52,662
Other comprehensive income, net of tax	—	—	—	99	—	99
Net loss	—	—	—	—	(116,914)	(116,914)
Balance, December 31, 2024	6,238,799	$ 13	$ 553,623	$ 22	$ (456,994)	$ 96,664
Issuance of common stock upon vesting of RSUs, net of shares withheld for taxes, exercise of stock options, and issuance of common stock under ESPP	115,297	—	1,926	—	—	1,926
Issuance of common stock from at-the-market sales agreement	1,910,861	1	70,105	—	—	70,106
Stock-based compensation expense	—	—	18,912	—	—	18,912
Issuance of common stock from private placement, net of issuance costs	13,876,032	2	149,883	—	—	149,885
Issuance of common stock from exercise of pre-funded warrants	5,079,207	1	86,190	—	—	86,191
Issuance of common stock from underwritten offering, net of issuance costs	11,500,000	1	107,965	—	—	107,966
Other comprehensive income, net of tax	—	—	—	19	—	19
Net loss	—	—	—	—	(695,981)	(695,981)
Balance, December 31, 2025	38,720,196	18	988,604	41	(1,152,975)	(164,312)

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

VOR BIOPHARMA INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)	Year Ended December 31, 2025	2024
Cash flows from operating activities		
Net loss	$ (695,981)	$ (116,914)
Adjustments to reconcile net loss to net cash used in operations:		
Depreciation expense	2,887	3,528
Non-cash lease expense	5,647	5,041
Stock-based compensation	18,912	9,847
Transaction costs for issuance of pre-funded warrants	643	—
Change in fair value of warrant liabilities	334,358	—
Interest amortization on marketable securities	(67)	(1,149)
Loss (gain) on sale of property and equipment	3,303	(18)
Issuance of pre-funded warrants in connection with the acquisition of in-process research and development	177,377	—
Changes in operating assets and liabilities:		
Operating lease liabilities	(2,406)	(3,830)
Prepaid expenses and other current assets	1,684	(835)
Accounts payable and accrued liabilities	10,930	2,241
Other assets	—	2,429
Net cash used in operating activities	(142,713)	(99,660)
Cash flow from investing activities		
Purchases of marketable securities	(53,660)	(9,914)
Proceeds from maturities of marketable securities	5,000	107,000
Purchases of property and equipment	(941)	(229)
Proceeds from sales of property and equipment	799	—
Net cash (used in) provided by investing activities	(48,802)	96,857
Cash flow from financing activities		
Proceeds from the issuance of common stock from private placement	149,999	55,550
Proceeds from the issuance of common stock from underwritten public offering, net of underwriting discount and commissions	108,101	—
Proceeds from the issuance of pre-funded warrants from private placement	175,000	—
Payment of issuance costs related to underwritten public offering and private placements	(1,325)	(2,325)
Proceeds from the issuance of common stock from at-the-market sales agreement, net of issuance costs	70,106	275
Payment for tax withholdings upon vesting of restricted stock unit awards	(254)	(333)
Proceeds from stock option exercises and the issuance of shares under ESPP	2,180	225
Net cash provided by financing activities	503,807	53,392
Net increase in cash, cash equivalents and restricted cash equivalents	312,292	50,589
Cash, cash equivalents and restricted cash equivalents, beginning of period	$ 84,362	$ 33,773
Cash, cash equivalents and restricted cash equivalents, end of period	$ 396,654	$ 84,362
Supplemental disclosure of non-cash activities		
Right-of-use assets obtained in exchange for lease obligations	$ 3,079	$ —
Financing costs associated with the sale of common stock included in accounts payable and accrued expenses	$ 127	$ 563
Unrealized gain on available-for-sale securities	$ 19	$ 99
Issuance of pre-funded warrants in connection with the acquisition of in-process research and development	$ 177,377	$ —

A reconciliation of the cash, cash equivalents and restricted cash reported within the consolidated balance sheets that sum to the total of the same amounts shown in the statements of cash flows is as follows:

(in thousands)	For the Year Ended December 31, 2025	2024
Cash and cash equivalents	$ 396,486	$ 81,949
Restricted cash equivalents	168	2,413
Total cash, cash equivalents and restricted cash equivalents as shown on the statements of cash flows	$ 396,654	$ 84,362

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

VOR BIOPHARMA INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of the Business

Vor Biopharma Inc. (the "Company") is a clinical-stage company advancing telitacicept, a novel, dual-target recombinant fusion protein that inhibits both BLyS (BAFF) and APRIL—two key cytokines involved in B cell survival and autoantibody production. This dual-target mechanism reduces autoreactive B cells and autoantibody production, key drivers of autoimmune pathology. The Company is headquartered in Boston, Massachusetts. The Company was incorporated on December 30, 2015.

Risks and Uncertainties

The Company is subject to a number of risks common to development stage companies in the biotechnology industry, including, but not limited to, risks of failure of clinical trials, dependence on key personnel, protection of proprietary technology, reliance on third party organizations, uncertainty of obtaining regulatory approval for any product candidate that it may develop, development by competitors of technological innovations, compliance with government regulations, adverse macroeconomic conditions, and the need to obtain additional financing.

Liquidity and Capital Resources

As of December 31, 2025, the Company had $455.2 million of cash, cash equivalents and marketable securities and an accumulated deficit of $1,153.0 million. The Company expects that its existing cash, cash equivalents and marketable securities will be sufficient to allow the Company to fund its current planned operations through at least a period of one year after the date the financial statements are issued. The Company anticipates that it will continue to incur significant operating losses for the next several years as it continues to develop its product candidate. As a result, the Company's continued operations are dependent on its ability to raise additional funding. If the Company is unable to obtain additional funding on a timely basis, it may be forced to significantly curtail, delay, or discontinue one or more of its planned research or development programs or be unable to expand its operations.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") or an Accounting Standards Update ("ASU") issued by the Financial Accounting Standards Board ("FASB"). The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany accounts and transactions are eliminated upon consolidation.

September 2025 Reverse Stock Split

On August 25, 2025, the Company's stockholders approved a proposal to authorize the Company's board of directors to amend the Company's Amended and Restated Certificate of Incorporation to effect a reverse stock split. The Board approved the reverse stock split on August 27, 2025 and, on September 18, 2025, the Company effected a 1-for-20 reverse stock split of its common stock. The par value and the number of authorized shares of common stock were not adjusted as a result of the reverse stock split. All share and per share amounts for all periods presented in these consolidated financial statements and the notes thereto have been adjusted retroactively, where applicable, to reflect the effect of this reverse stock split.

Use of Estimates

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

process often may yield a range of potentially reasonable estimates and management must select an amount that falls within that range of reasonable estimates. Estimates are used in the following areas, among others: accrued expenses and stock-based compensation expense.

Segments

Operating segments are defined as components of an enterprise for which separate and discrete information is available for evaluation by the chief operating decision maker in deciding how to allocate resources and assess performance. The Company has one operating segment. The Company's chief operating decision maker, its Chief Executive Officer, manages the Company's operations on an aggregate basis for the purpose of allocating resources. Refer to Note 15 for more information.

Cash and Cash Equivalents

The Company considers highly-liquid investments purchased with an original maturity date of ninety days or less from the date of purchase to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market funds. Cash equivalents are stated at cost, which approximates market value.

Marketable Securities

Investments in marketable debt securities are classified as available-for-sale. Marketable securities with maturities beyond one year may be classified as short-term based on their highly liquid nature and because such securities represent an investment of cash that is available for current operations.

Available-for-sale marketable securities are reported at fair value at each balance sheet date. Amortization and accretion of premiums and discounts are recorded in interest income. Realized gains and losses are included as a component of other income (expense), net in the consolidated statements of operations.

The Company evaluates its marketable securities with unrealized losses for impairment. When assessing marketable securities for unrealized declines in value, the Company considers whether the decline in value is related to a credit loss or non-credit loss. For credit losses, the Company reduces the marketable security to fair value through an allowance for credit losses recorded to the balance sheet and corresponding charge to the statement of operations. The allowance for credit losses and corresponding impairment charge is adjusted each period for changes in fair value. For non-credit losses, the Company reduces the marketable security to fair value through a charge to the statement of operations and comprehensive loss, reported as a component of accumulated other comprehensive income (loss) in stockholders' equity (deficit). No credit losses were recorded during the periods presented.

Restricted Cash Equivalents

The Company had $0.2 million and $2.4 million of restricted cash equivalents in the form of a letter of credit related to a lease at December 31, 2025 and 2024, respectively.

Comprehensive Income (Loss)

Comprehensive loss includes net loss, as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders.

Concentrations of Credit Risk

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents, restricted cash equivalents and marketable securities. The Company maintains deposits in accredited financial institutions in excess of federally insured limits. The Company deposits its cash in financial institutions that it believes have high credit quality and has not experienced any losses on such accounts and does not believe it is exposed to any unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company's marketable securities may consist of money market funds and marketable debt securities, including corporate bonds and U.S. Treasury securities. The Company's investment policy limits instruments to investment grade securities with high credit quality issuers with the objective to preserve capital and to maintain liquidity until the funds can be used in business operations.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

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

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

Property and Equipment, Net

Property and equipment, net is recorded at cost less accumulated depreciation. Depreciation expense is recorded using the straight-line method over the estimated useful life of the related asset, which are as follows:

	Estimated Useful Life
Computer equipment	3 years
Manufacturing equipment	5 years
Furniture and equipment	5 years
Laboratory equipment	5 years
Leasehold improvements	Shorter of remaining lease term or useful life

Purchased assets that are not yet in service are recorded to construction-in-process and no depreciation expense is recorded. Once they are placed in service, they are reclassified to the appropriate asset class. When assets are retired or otherwise disposed of, the assets and related accumulated depreciation are eliminated from the accounts and any resulting gain or loss is reflected in the Company's consolidated statements of operation and comprehensive loss. Expenditures for maintenance and repairs are expensed as incurred.

Impairment of Long-Lived Assets

Long-lived assets consist of property, equipment and right-of-use assets. The Company continually evaluates whether events or circumstances have occurred that indicate that the estimated remaining useful life of its long-lived assets may warrant revision or that the carrying value of these assets may not be recoverable. If circumstances require that a long-lived asset or asset group be tested for impairment, the Company first compares the estimated undiscounted future cash flows expected to result from the use or disposition of that asset or asset group to its carrying amount. If the carrying amount of the long-lived asset or asset group is not recoverable on an undiscounted cash flow basis, an impairment loss would be recognized to the extent the carrying value exceeds its fair value. Fair value is determined through various valuation techniques including discounted cash flow models, quoted market prices and third-party independent appraisals, as considered necessary. The Company did not recognize any impairment loss in the year ended December 31, 2025.

Leases

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease. Leases with a term greater than one year are recognized on the consolidated balance sheet as a right-of-use ("ROU")

asset and current and non-current lease liabilities, as applicable. The Company has made an accounting policy election, known as the short-term lease recognition exemption, which allows the Company to not recognize ROU assets and lease liabilities that arise from short-term leases (12 months or less). The Company has applied this election to all classes of underlying assets. The Company typically only includes an initial lease term in its assessment of a lease arrangement. Options to renew or options to cancel a lease are not included in the Company's assessment unless there is reasonable certainty that the Company will renew or will not cancel, respectively. The Company monitors its material leases on a quarterly basis.

Operating lease liabilities and their corresponding ROU assets are recorded based on the present value of future lease payments over the expected remaining lease term. Lease cost for operating leases is recognized on a straight-line basis over the lease term as an operating expense. Certain adjustments to the ROU asset may be required for items such as lease prepayments or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate, which reflects the fixed rate at which the Company could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment.

The Company has elected to account for lease and non-lease components together.

Research and Development

Research and development expenses include costs directly attributable to the conduct of the Company's research and development programs.

Expenditures relating to research and development are expensed in the period incurred. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made. The cost of materials for a research and development activity that have an alternative future use is capitalized when the materials are acquired and recognized as expense as consumed. The costs of materials that were acquired for a particular research and development activity and have no alternative future use are expensed in the period acquired.

Costs incurred in obtaining licenses are recognized as research and development expense as incurred if the license has no alternative use.

Accrued Research and Development Expenses

The Company has entered into various research and development related contracts, including contracts with third-party contract research organizations and contract manufacturing organizations. These agreements are cancelable, and related costs are recognized as research and development expenses as incurred. The Company records accrued liabilities for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes the progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates may be required to determine the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. To date, the Company's historical accrual estimates have not been materially different from the actual costs.

Warrants

The Company accounts for warrants to purchase its common stock as either equity-classified or liability-classified instruments based on an assessment of the warrant's specific terms and applicable authoritative guidance in ASC Topic 480, *Distinguishing Liabilities from Equity* ("ASC 480") and ASC Topic 815, *Derivatives and Hedging* ("ASC 815"). The assessment considers whether warrants are freestanding financial instruments pursuant to ASC 480, meet the liability classification requirements pursuant to ASC 480, and whether the warrants meet all of the requirements for equity classification under ASC 815, including whether the warrants are indexed to the Company's own stock, among other conditions for equity classification. This assessment, which requires the use of professional judgment, is conducted at the time of warrant issuance and as of each subsequent period end date while the warrants are outstanding.

Warrants classified as equity are recorded at fair value as of the date of issuance on the Company's consolidated balance sheets and no further adjustments to their initial valuation will be subsequently made as long as they remain equity-classified. Warrants classified as liabilities are recorded on the Company's consolidated balance sheets at their fair value on the date of issuance and are revalued at each subsequent balance sheet date until such

instruments are exercised or expired, or meet the criteria to become equity-classified, with any changes in the fair value between reporting periods recorded as a component of other income (expense), net on the consolidated statement of operations and comprehensive loss.

As of December 31, 2025, the Company has both equity-classified and liability-classified warrants outstanding.

Stock-Based Compensation Expense

The Company accounts for stock-based compensation under the provisions of ASC 718-10, *Compensation—Stock Compensation* ("ASC 718-10"), which requires all stock-based payments to employees, non-employees and directors, including grants of stock options and restricted stock units, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values on the date of grant over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. Generally, the Company issues awards with only service-based vesting conditions and records the expense for these awards using the ratable method. The Company classifies stock-based compensation expense in the same manner in which the award recipient's payroll or service provider's costs are classified. Stock-based payments that contain performance conditions are recognized when such conditions are probable of being achieved.

The fair value of each restricted common stock award and each restricted stock unit award is estimated on the date of grant based on the fair value of the Company's common stock on that same date.

The fair value of each option grant is estimated on the date of grant using the Black-Scholes option pricing model which requires inputs based on certain subjective assumptions, including the following:

- *Expected Term*—The expected term represents the period that the stock-based awards are expected to be outstanding. The Company uses the simplified method to determine the expected term, which is based on the average of the time-to-vesting and the contractual life of the options.
- *Expected Volatility*—Because the Company does not have sufficient trading history for its common stock as of December 31, 2025, the expected volatility was estimated based on the average volatility for comparable publicly traded biotechnology companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on the similar size, stage in life cycle or area of specialty. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.
- *Risk-Free Interest Rate*—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of the awards.
- *Dividend Yield*—The Company has never paid dividends on its common stock and has no plans to pay dividends on its common stock. Therefore, the Company used an expected dividend yield of zero.

Income Taxes

The Company accounts for income taxes using the asset and liability approach. Deferred tax assets and liabilities represent future tax consequences of temporary differences between the financial statement carrying amounts and the tax basis of assets and liabilities and for loss carryforwards using enacted tax rates expected to be in effect in the years in which the differences reverse. A valuation allowance is established to reduce deferred tax assets to the amounts expected to be realized. The Company also recognizes a tax benefit from uncertain tax positions only if it is "more likely than not" that the position is sustainable based on its technical merits. The Company accounts for interest or penalties related to uncertain tax positions as part of its provision for income taxes. To date, the Company has not incurred interest and penalties related to uncertain tax positions. Should such costs be incurred, they would be classified as a component of provision for income taxes.

Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted-average number of common shares outstanding during the reporting period, without consideration for potentially dilutive securities. Diluted net loss per share is computed by dividing net loss by the weighted-average number of common shares and potentially dilutive securities outstanding during the periods. For purposes of the diluted net loss per share calculation, warrants, restricted stock units and stock options considered to be potentially dilutive securities were 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 were the same for all reporting periods presented.

Restructuring Costs

Employee severance costs are recorded based on whether the termination benefits are provided under an on-going benefit arrangement or under a one-time benefit arrangement. The Company accounts for on-going termination benefit arrangements, such as those arising from employment agreements, applicable regulations or past practices, in accordance with ASC 712, *Compensation-Nonretirement Postemployment Benefits*. Under ASC 712, liabilities for post-employment benefits are recorded at the time the obligations are probable of being incurred and can be reasonably estimated. The Company accounts for one-time employment benefit arrangements in accordance with ASC 420, *Exit or Disposal Cost Obligations*. One-time termination benefits are expensed at the date the entity notifies the employee, unless the employee must provide future service over a period extending past the minimum notification period, in which case the benefits are expensed ratably over the future service period. Other associated costs are recognized in the period in which the liability is incurred. Refer to Note 16 for additional information on the severance expense that the Company recognized for employees terminated in connection with the Restructuring Plan (as defined below).

Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued and adopted by the Company as of the specified effective date. Unless otherwise discussed, the impact of recently issued standards that are not yet effective are not expected to have a material impact on the Company's consolidated financial statements upon adoption. Under the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act"), the Company meets the definition of an emerging growth company and has elected to take advantage of the extended transition period for complying with certain new or revised accounting standards pursuant to Section 107(b) of the JOBS Act.

Recently Issued Accounting Pronouncements Not Yet Adopted

In December 2023, the FASB issued ASU 2023-09, Income Taxes (Topic 740): Improvements to Income Tax Disclosures ("ASU 2023-09"). ASU 2023-09 requires more detailed income tax disclosures, requiring entities to disclose disaggregated information about their effective tax rate reconciliation as well as expanded information on income taxes paid by jurisdiction. The disclosure requirements will be applied on a prospective basis, with the option to apply them retrospectively. This update is effective beginning with the Company's 2026 fiscal year annual reporting period. The Company is currently evaluating the impact that the adoption of this standard will have on its consolidated financial statements and disclosures.

In November 2024, the FASB issued ASU 2024-03, Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses ("ASU 2024-03"). ASU 2024-03 improves disclosures surrounding a public business entity's expenses by requiring more detailed information about the types of expenses included within commonly presented income statement captions. The update is effective beginning with the Company's 2027 fiscal year annual reporting period, with early adoption permitted. The Company is currently evaluating the impact that the adoption of this standard will have on its consolidated financial statements and disclosures.

In December 2025, the FASB issued ASU 2025-11, Interim Reporting (Topic 270) ("ASU 2025-11"). ASU 2025-11 provides clarity about current interim reporting requirements to help entities determine whether disclosures not specified in Topic 270 should be provided in interim reporting periods. The update is effective beginning with the Company's 2028 fiscal year annual reporting period, with early adoption permitted. The Company does not expect the update to have a material impact on the consolidated financial statements.

3. Marketable Securities

The amortized cost and estimated fair value of marketable securities, by contractual maturity are as follows:

(in thousands)	December 31, 2025 Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Maturing in one year or less				
U.S. Treasuries	$ 25,772	$ 16	$ —	$ 25,788
U.S. Treasury Bills	$ 13,636	$ 6	—	$ 13,642
Maturing after one year through five years				
U.S. Treasuries	$ 19,273	$ 19	—	$ 19,292
Total	$ 58,681	$ 41	$ —	$ 58,722

(in thousands)	December 31, 2024 Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Maturing in one year or less				
U.S. Treasuries	$ 4,981	$ 12	$ —	$ 4,993
Maturing after one year through five years				
U.S. Treasuries	4,974	10	—	4,984
Total	$ 9,955	$ 22	$ —	$ 9,977

The Company did not have any individual securities in an unrealized loss position as of December 31, 2025 or 2024. The Company did not record any impairments to marketable securities or reserves for credit losses related to its marketable debt securities during the years ended December 31, 2025 and December 31, 2024.

4. Fair Value Measurements

The following tables present information about the Company's financial assets and liabilities measured at fair value on a recurring basis:

(in thousands)	December 31, 2025 Level 1	Level 2	Level 3	Total
Cash equivalents				
Money market funds	$ 392,803	$ —	$ —	$ 392,803
U.S. Treasury bills	—	3,434	—	3,434
Total cash equivalents	392,803	3,434	—	396,237
Marketable securities				
U.S. Treasury bills	—	13,642	—	13,642
U.S. Treasuries	—	45,080	—	45,080
Total marketable securities	—	58,722	—	58,722
Restricted cash equivalents				
Money market funds	168	—	—	168
Liabilities				
Warrant liabilities	—	600,547	—	600,547

(in thousands)	December 31, 2024			
	Level 1	Level 2	Level 3	Total
Cash equivalents				
Money market funds	$ 81,718	$ —	$ —	$ 81,718
Marketable securities				
U.S. Treasuries	—	9,977	—	9,977
Total marketable securities	—	9,977	—	9,977
Restricted cash equivalents				
Money market funds	2,413	—	—	2,413

The fair value of the Company's cash equivalents and restricted cash equivalents is based on quoted market prices in active markets with no valuation adjustment. The fair values of marketable securities and warrant liabilities are determined based on observable market inputs. During the years ended December 31, 2025 and 2024, there were no transfers between levels.

Prepaid expenses, accounts payable and accrued expenses are stated at their respective historical carrying values which approximate fair value due to their short-term nature.

5. Property and Equipment, Net

Property and equipment, net consisted of the following:

(in thousands)	December 31, 2025	December 31, 2024
Laboratory equipment	$ —	$ 9,625
Manufacturing equipment	—	7,082
Computer equipment	—	446
Furniture, fixtures and other	558	606
Construction in progress	—	36
Total	558	17,795
Less: Accumulated depreciation	(25)	(11,214)
Property and equipment, net	$ 533	$ 6,581

Depreciation expense for the years ended December 31, 2025 and 2024 was $2.9 million and $3.5 million, respectively. In connection with the Restructuring Plan (as defined below in Note 16, *Restructuring*), during the year ended December 31, 2025, the Company recognized a $3.3 million loss on disposal of certain long-lived assets including equipment and leasehold improvements which was recognized as research and development expense in the consolidated statements of operations and comprehensive loss.

6. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	December 31, 2025	December 31, 2024
Employee-related expenses	$ 3,066	$ 5,852
Professional fees	1,366	1,461
Clinical expenses	8,466	3,835
Manufacturing expenses	6,071	516
Research and development expenses	241	872
Other	554	356
Total accrued liabilities	$ 19,764	$ 12,892

7. Stockholders' Equity

Common Stock

As of December 31, 2025 and 2024, the Company's authorized capital stock included 800,000,000 and 400,000,000 shares of its $0.0001 par value common stock, respectively, and 10,000,000 shares of its $0.0001 par value preferred stock.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders provided, however, that, except as otherwise required by law, holders of common stock shall not be entitled to vote on any amendment to the Company's certificate of incorporation, as amended (the "Certificate of Incorporation"), that relates solely to the terms of one or more outstanding series of preferred stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the Certificate of Incorporation or pursuant to the Delaware General Corporation Law. Common stockholders are entitled to receive dividends, as may be declared by the Company's board of directors, if any, subject to the preferential dividend rights of the preferred stock. No dividends have been declared or paid as of and for either of the years ended December 31, 2025 and 2024.

At-the-Market Sales

During the years ended December 31, 2025 and 2024, the Company sold 1,910,861 and 12,454 shares of common stock, respectively, in at-the-market offerings at a weighted average price per share of $37.06 and $32.09, respectively, for aggregate net proceeds of $70.1 million and $0.3 million, respectively, after deducting commissions.

2024 Private Placement

On December 27, 2024, the Company entered into a purchase agreement with certain institutional investors pursuant to which the Company issued and sold in a private placement an aggregate of (i) 2,793,562 shares of the Company's common stock and (ii) warrants to purchase up to 3,491,953 shares of the Company's common stock (the "2024 Warrants") at the closing of the private placement on December 30, 2024. Net proceeds from the private placement were $52.7 million, after deducting placement fees and issuance costs payable by the Company.

The 2024 Warrants have an exercise price of $16.76 per share and are immediately exercisable, subject to certain limitations on exercise set forth in the 2024 Warrants. The 2024 Warrants will terminate on December 30, 2031.

The Company determined that the 2024 Warrants are freestanding instruments that do not meet the definition of a liability or derivative. The 2024 Warrants are indexed to the Company's common stock and meet all other conditions for equity classification and have continued to meet such conditions for all periods presented. Accordingly, the 2024 Warrants are classified as equity and accounted for as a component of additional paid-in capital. The Company also determined that the 2024 Warrants should be included in the determination of diluted net loss per share if their impact is dilutive. However, they are not included within diluted net loss per share for the years ended December 31, 2025 or 2024 as their effect would be antidilutive.

As of December 31, 2025, none of the 2024 Warrants have been exercised.

June 2025 Private Placement

On June 25, 2025, the Company entered into a purchase agreement with certain institutional investors (collectively, the "2025 Purchasers"), pursuant to which the Company issued and sold to the 2025 Purchasers in a private placement pre-funded warrants to purchase up to an aggregate of 34,999,999 shares of the Company's common stock (the "2025 PIPE Warrants") at the closing on June 27, 2025. Net proceeds from the private placement were $174.4 million, after deducting issuance costs payable by the Company. In addition to the 2025 PIPE Warrants, on June 25, 2025 the Company issued a warrant to purchase up to 16,000,000 shares of the Company's common stock as partial consideration for the Telitacicept License Agreement (as defined below) to a subsidiary of RemeGen Co., Ltd. ("Remegen") (the "RemeGen Warrant"). Refer to Note 10 for additional

information on the license arrangement. The 2025 PIPE Warrants and RemeGen Warrant are collectively referred to as the 2025 Warrants.

The 2025 Warrants have an exercise price of $0.002 per share and became exercisable upon stockholder approval of the issuance of the underlying shares and an amendment to the Certificate of Incorporation to increase the number of authorized shares, subject to certain limitations on exercise set forth in the 2025 Warrants. The 2025 Warrants do not expire.

Upon issuance, the 2025 Warrants were liability-classified as they are not considered indexed to the Company's common stock. The 2025 Warrants are measured at fair value each period with changes in fair value presented within the consolidated statements of operations and comprehensive loss. The valuation of the 2025 Warrants is classified within Level 2 of the fair value hierarchy due to the use of observable market inputs, primarily the quoted price of the Company's common stock underlying the warrants. The initial carrying value of the 2025 PIPE Warrants and the RemeGen Warrant at issuance was $175.0 million and $177.4 million, respectively. Issuance costs related to the 2025 PIPE Warrants were expensed as incurred.

The Company determined the 2025 Warrants should be included in the determination of diluted net loss per share if their impact is dilutive. However, they are not included within diluted net loss per share for the year ended December 31, 2025 as the effect would be antidilutive. Because the 2025 Warrants are liability-classified, they are excluded from basic net loss per share until exercised.

During the year ended December 31, 2025, 5,079,640 of the 2025 Warrants were exercised for an immaterial amount of net proceeds, with certain of the exercises completed on a cashless basis. The remaining 45,920,359 outstanding 2025 Warrants had a fair value of $600.5 million as of December 31, 2025.

November 2025 Public Offering

On November 10, 2025, the Company entered into an underwriting agreement relating to the issuance and sale in a public offering of 11,500,000 shares of the Company's common stock, including 1,500,00 shares purchased by the underwriters under a 30-day option to purchase additional shares (the "November 2025 Offering") at a public offering price of $10.00 per share. The net proceeds to the Company from the November 2025 Offering were $107.7 million after deducting the underwriting discounts and commissions and offering expenses.

December 2025 Private Placement

On December 15, 2025, the Company entered into a securities purchase agreement with certain investors pursuant to which the Company, in a private placement, issued and sold an aggregate of 13,876,032 shares of common stock, at a price per share of $10.81, for net proceeds of $149.9 million after deducting offering expenses (the "December 2025 Private Placement").

8. Stock-Based Compensation

Stock Incentive Plans

In December 2015, the Company's board of directors adopted and approved the 2015 Stock Incentive Plan (as amended to date, the "2015 Plan"). The 2015 Plan provided for the granting of incentive stock options, non-statutory stock options, restricted stock awards and other stock-based awards to eligible employees, officers, directors, consultants and advisors as determined by the Company's board of directors.

In February 2021, the Company's board of directors adopted and stockholders approved the 2021 Equity Incentive Plan (the "2021 Plan"). The 2021 Plan became effective on February 5, 2021, following which no further grants were or will be made under the 2015 Plan. In March 2024, the Company's board of directors adopted an amendment and restatement of the 2021 Plan, which the Company's stockholders approved in May 2024. The 2021 Plan provides for the grant of stock options, non-statutory stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance stock awards and other forms of stock compensation to the Company's employees, consultants and directors.

The number of shares of the Company's common stock reserved for issuance under the 2021 Plan will automatically increase on January 1 of each year through January 1, 2035, by 4.0% of the total number of shares of

as common stock outstanding on December 31 of the preceding calendar year. Any grants that expire or are canceled, terminated, forfeited, fail to vest, or are withheld to satisfy a tax withholding obligation are allowed to be reissued under 2021 Plan. As of December 31, 2025, the Company had 433,804 shares of its common stock available for future issuance under the 2021 Plan.

In August 2023, the Company's board of directors adopted the Company's 2023 Inducement Plan (the "2023 Inducement Plan") pursuant to which the Company reserved 3,500,000 shares of common stock for issuance under the 2023 Inducement Plan. The 2023 Inducement Plan provides for the grant of non-statutory stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance awards and other forms of stock-based compensation to eligible individuals. In accordance with Nasdaq Marketplace Rule 5635(c)(4), awards under the 2023 Inducement Plan may only be made to individuals not previously employees or directors of the Company (or following such individuals' bona fide period of non-employment with the Company), as an inducement material to the individuals' entry into employment with the Company. Awards granted under the 2023 Inducement Plan must be approved by either a majority of the Company's independent directors or the compensation committee of the Company's board of directors. In June 2025, the Company's board of directors approved an amendment to the 2023 Inducement Plan to reserve an additional 7,511,334 shares of common stock for issuance under the 2023 Inducement Plan. As of December 31, 2025, the Company had 2,539,361 shares of its common stock available for future issuance under the 2023 Inducement Plan.

February 2025 Option Repricing

On February 3, 2025, the Company's board of directors approved a stock option repricing (the "February 2025 Option Repricing") whereby the exercise price of certain outstanding options to purchase shares of the Company's common stock was reduced to $26.80 per share. The repricing applied to options to purchase shares of the Company's common stock held by continuing employees as of February 3, 2025 that had an exercise price per share greater than $26.80; provided that holders of repriced options must remain in continuous service with the Company through February 3, 2026 or, if earlier, a change in control of the Company or 30 days prior to the applicable repriced option's original expiration date. If any such repriced option is exercised prior to such time, the exercise price per share will be the original exercise price per share, and not the repriced exercise price. The total number of shares underlying all repriced options was 337,809. The repriced options previously had exercise prices ranging from $27.20 to $899.20 per share. Management determined that the February 2025 Option Repricing represents a modification of share-based awards and calculated incremental compensation cost of $1.9 million resulting from the modification. However, as the conditions of the modified terms were not expected to be met, the Company has not recognized any incremental compensation cost associated with the February 2025 Option Repricing.

December 2025 Equity Award Cancellation and Replacement

On December 5, 2025, the Company executed a cancellation of the Chief Financial Officer's award of 694,137 restricted stock units ("RSUs"), which was granted on July 9, 2025, and concurrently replaced it with a grant of 1,388,274 options on the date of cancellation. The cancellation and concurrent replacement grant was treated as an award modification. The incremental compensation cost resulting from this modification was $2.4 million, of which $0.1 million was recognized in the year ended December 31, 2025.

December 2025 Option Repricing

On December 5, 2025, the Company's board of directors approved a stock option repricing (the " December 2025 Option Repricing") whereby the exercise price of certain outstanding options to purchase shares of the Company's common stock was reduced to $8.18 per share. The repricing applied to options to purchase shares of the Company's common stock held by continuing employees as of December 5, 2025 that had an exercise price per share greater than $8.18; provided that holders of repriced options must remain in continuous service with the Company through December 5, 2027 or, if earlier, a change in control of the Company or 30 days prior to the applicable repriced option's original expiration date. If any such repriced option is exercised prior to such time, the exercise price per share will be the original exercise price per share, and not the repriced exercise price. The total number of shares underlying all repriced options was 6,582,767. The repriced options previously had exercise prices ranging from $17.80 to $47.60 per share. Management determined that the December 2025 Option Repricing represents a modification of share-based awards and calculated incremental compensation cost of $7.0 million resulting from the modification. The Company recognized $0.1 of this incremental expense in the year ended December 31, 2025; the remaining incremental expense will be fully recognized by 2029.

Stock Options

The Company's stock options generally vest over 48 months with 25% vesting after one year followed by ratable monthly vesting over three years and have a contractual term of 10 years. The weighted-average assumptions used principally in determining the fair value of options granted are presented in the table below. The below assumptions reflect those used to determine the original grant date fair value for options granted, and do not reflect the impact of the modifications described above.

	Year Ended December 31,	
	2025	2024
Expected term (in years)	6.0	6.0
Expected volatility	98.9%	88.9%
Risk-free interest rate	3.9%	4.1%
Dividend yield	—	—

The following table summarizes the Company's stock option activity for the year ended December 31, 2025:

	Shares	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2024	457,152	$ 104.41	6.99	$ 211
Granted	6,837,865	21.11		
Vested and exercised	(61,189)	34.33		
Forfeited	(438,733)	44.45		
Expired	(186,829)	136.36		
Outstanding at December 31, 2025	6,608,266	10.32	9.39	$ 31,429
Exercisable at December 31, 2025	181,128	$ 82.86	1.65	$ 56

*Weighted-average exercise price for shares outstanding at the end of the period reflect the exercise price resulting from the February and December 2025 Option Repricings. Each of the other weighted-average exercise prices included above reflect the original grant date exercise price for such grants. None of the options exercisable at year-end were subject to the repricings.

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the common stock as of the respective date. The intrinsic value for options outstanding at the end of the period reflects the impact of the December 2025 Option Repricing.

The weighted-average grant-date fair value of stock options granted during the years ended December 31, 2025 and 2024 was $15.09 and $29.60 per share, respectively. The weighted-average grant date fair value for options granted in 2025 reflects the original grant date fair value and does not reflect the impact of any repricing. The weighted-average fair value of the repriced options was $5.54 immediately prior to the repricing, and $6.60 immediately after the effect of the repricing. As of December 31, 2025, total unrecognized compensation expense related to stock options was $113.4 million which is expected to be recognized over a weighted-average period of 3.5 years. There were 61,189 stock options exercised during the year ended December 31, 2025.

Restricted Stock Units

As of December 31, 2025, there were 186,325 restricted stock units outstanding under the 2021 Plan and the 2023 Inducement Plan. The following table summarizes the Company's unvested restricted stock unit activity for the year ended December 31, 2025:

	Shares	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2024	66,046	$ 58.35
Granted	981,837	46.22
Vested	(30,638)	51.56
Forfeited or cancelled	(830,920)	49.41
Unvested at December 31, 2025	186,325	$ 35.40

As of December 31, 2025, total unrecognized compensation expense related to the unvested restricted stock units was $5.9 million, which is expected to be recognized over a weighted average period of 3.6 years.

2021 Employee Stock Purchase Plan

In February 2021, the Company's board of directors adopted and stockholders approved the 2021 Employee Stock Purchase Plan (the "ESPP"). The ESPP became effective on February 5, 2021. The number of shares of common stock reserved for issuance under the ESPP will automatically increase on January 1 of each year through January 1, 2031, by the lesser of (i) 1% of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, and (ii) 90,000 shares. If purchase rights granted under the ESPP terminate without having been exercised, the shares of common stock not purchased under such purchase rights will again become available for issuance under the ESPP. As of December 31, 2025, the Company had 115,932 shares of its common stock available for future issuance under the ESPP.

The ESPP permits eligible employees to purchase common stock through accumulated payroll deductions at a purchase price equal to 85% of the lesser of the market value of the common stock at the beginning of the 6-month offering period or on the purchase date. During the years ended December 31, 2025 and 2024, the Company issued 33,477 and 11,392 shares, respectively, with a weighted average purchase price per share of $2.91 and $19.60, respectively, which resulted in an immaterial amount of compensation expense.

Stock-Based Compensation

Stock-based compensation expense was allocated as follows:

	Year Ended December 31,	
(in thousands)	2025	2024
Research and development	$ 1,957	$ 4,728
General and administrative	16,955	5,119
Total stock-based compensation expense	$ 18,912	$ 9,847

9. Leases

Cambridgepark Lease

In December 2019, the Company entered into a lease agreement for office and laboratory space (the "Cambridgepark Lease") in Cambridge, Massachusetts with PPF Off 100 Cambridge Park Drive, LLC (the "Landlord"). During 2021 and 2022, the Company entered into various lease amendments with the Landlord to obtain additional leased space (the "Lease Amendments").

In connection with the Restructuring Plan, the Company entered into an early termination agreement with the Landlord on June 20, 2025, pursuant to which the parties agreed to terminate the lease, effective August 4, 2025. Per the terms of such agreement, the Company paid a non-refundable termination fee in the amount of $8.5 million to the Landlord. The early termination was treated as a lease modification for accounting purposes. As a result of the modification, the Company remeasured the lease liability and recognized a corresponding adjustment to the right-of-use asset as of the date of the modification. Additionally, as of June 30, 2025, the space was determined to be

abandoned, thus the Company accelerated amortization of the right-of-use asset and de-recognized any remaining balances.

In conjunction with the Cambridgepark Lease, the Company was required to execute an irrevocable standby letter of credit of $2.4 million for the benefit of the Landlord. The funds were released to the Company during the quarter ended September 30, 2025.

Boylston Lease

In August 2025, the Company entered into a lease agreement for office space ("Boylston Lease") with 500 Boylston & 222 Berkeley Owner (DC) LLC (the "Boylston Landlord"). The commencement date of the lease was September 1, 2025, and the Boylston Lease will expire on August 31, 2031, unless terminated earlier in accordance with the lease agreement. The Company has the option to extend the term for one additional five-year period.

Payments due associated with the Boylston Lease include both fixed and variable payments. Total fixed lease payments under the lease agreement are $3.8 million. Variable payments relate to the Company's share of the Boylston Landlord's operating costs associated with the underlying assets and are recognized when the event on which those payments are assessed occurs. The Boylston Lease does not contain a residual value guarantee.

In conjunction with the Boylston Lease, the Company was required to execute an irrevocable standby letter of credit of $0.2 million for the benefit of the Boylston Landlord. As of September 30, 2025, the funds securing the letter of credit were presented as restricted cash equivalents on the consolidated balance sheets.

The elements of lease expense were as follows:

	Year Ended December 31,	
(in thousands)	**2025**	**2024**
Operating lease cost	$ 15,278	$ 7,804
Variable lease cost	1,637	2,653
Total lease cost	$ 16,915	$ 10,457

Amounts reported in the consolidated balance sheets and the weight-average lease term and discount rate information were as follows:

(in thousands except weighted-average amounts)	**December 31, 2025**	**December 31, 2024**
Assets		
Operating lease right-of-use assets	$ 2,936	$ 35,007
Liabilities		
Operating lease liabilities, current	$ 280	$ 4,215
Operating lease liabilities, non-current	2,720	27,615
Total lease liabilities	$ 3,000	$ 31,830
Weighted-Average Lease Term and Discount Rate		
Weighted-average remaining lease term (years)	5.7	5.7
Weighted-average discount rate	6.7%	8.2%

The following table represents other lease activity:

	Year Ended December 31,	
(in thousands)	**2025**	**2024**
Other Information		
Cash paid for amounts included in the measurement of lease liabilities		
Operating cash flows for operating leases	$ 12,030	$ 6,591

Future lease payments for noncancelable leases as of December 31, 2025 were as follows:

(in thousands)	December 31, 2025
2026	$ 470
2027	586
2028	703
2029	717
2030	731
Thereafter	434
Total lease payments	$ 3,641
Less: imputed interest	(641)
Present value of lease liabilities	$ 3,000

10. Significant Agreements

Telitacicept License Agreement

On June 25, 2025, the Company and RemeGen entered into a license agreement (the "Telitacicept License Agreement") granting the Company exclusive rights to develop and commercialize RemeGen's proprietary fusion protein compound, telitacicept (the "Licensed Compound"), outside of the People's Republic of China, Hong Kong, Macau and Taiwan (collectively, "Greater China"). RemeGen retains all rights to the Licensed Compound in Greater China. Under the Telitacicept License Agreement, the Company received an exclusive (even as to RemeGen) license under RemeGen's patents and know-how to exploit, develop and commercialize the Licensed Compound in all territories other than Greater China, with the right to grant sublicenses. The Company also received a non-exclusive license to manufacture the Licensed Compound and any resulting licensed products ("Licensed Products") worldwide solely for use in the licensed territory. The Company is responsible for all development, regulatory and commercialization activities and costs in the licensed territory, including the conduct of clinical trials and regulatory submissions. The Telitacicept License Agreement established a joint steering committee ("JSC") to oversee the ongoing development and commercialization of telitacicept. The JSC comprises an equal number of senior-level executives from each party; however, final decision-making authority as it relates to the development and commercialization of the Licensed Compound outside Greater China lies with the Company.

As consideration for the rights granted, the Company agreed to make an upfront cash payment in the amount of $45.0 million and issued the RemeGen Warrant to a subsidiary of RemeGen, which was initially valued at $177.4 million. Refer to Note 7 for details on the terms of the RemeGen Warrant. The $45.0 million cash payment due to RemeGen was paid during the quarter ended September 30, 2025. The Company incurred $0.2 million in transaction costs related to the Telitacicept License Agreement during the year ended December 31, 2025.

The Company accounted for the Telitacicept License Agreement as an asset acquisition as substantially all of the value received was concentrated in the Licensed Compound which does not have an alternate future use as it is not yet approved for commercial sale in the licensed territory. Accordingly, the Company recognized a $222.6 million charge to research and development expense on the consolidated statement of operations and comprehensive loss during the year ended December 31, 2025 associated with this asset acquisition. The Company's accounting policy is to classify the cost of asset acquisitions as an operating cash outflow on the consolidated statement of cash flows.

Pursuant to the terms of the Telitacicept License Agreement, RemeGen is eligible to receive up to $330.0 million in regulatory milestone payments and up to $3.775 billion in sales milestone payments. In addition, RemeGen is entitled to receive tiered royalties on net sales of the Licensed Products in the licensed territory, ranging from high single digit to mid-teen percentages of net sales, subject to customary reductions. If the Company enters into a sublicense or divests rights to the Licensed Products prior to a specified development event and other than in connection with a change of control, RemeGen is entitled to receive a single digit percentage of certain net proceeds from such transactions. The Telitacicept License Agreement also provides for technology transfer, mutual

indemnification and confidentiality. As of December 31, 2025, no milestone or royalty payments have been accrued for as the consideration is not yet payable per the terms of the Agreement.

The Telitacicept License Agreement may be terminated, in its entirety or on a region-by-region basis, by either party for material breach (subject to cure periods and dispute resolution) or insolvency of the other party, by the Company for convenience with advance notice, or by RemeGen if the Company challenges the validity of licensed patents. Upon termination, all rights and licenses in the terminated region will revert to RemeGen, with a wind-down period for the Company to cease activities.

Sales of Intellectual Property

On August 8, 2025, the Company entered into an Asset Purchase Agreement pursuant to which the Company agreed to sell certain intellectual property related to the antibody drug conjugate known as VADC45 to Regeneron Pharmaceuticals, Inc. for $3.0 million in cash consideration. Additionally, on September 22, 2025, the Company entered into an Asset Purchase Agreement pursuant to which the Company agreed to sell certain intellectual property related to the Company's previous product candidates trem-cel and VCAR33 to SyzygyMed Inc. for $1.1 million in cash consideration.

11. Commitments and Contingencies

Legal Proceedings

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

12. Defined Contribution Benefit Plan

The Company maintains a defined contribution plan under Section 401(k) (the "401(k) Plan") of the Internal Revenue Code, as amended (the "Code"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pretax basis, as well as Roth post tax deferrals. The Company matches 100% of compensation amounts deferred up to the first 1% of an employee's compensation plus 50% of compensation amounts deferred between 1% and 6% of an employee's compensation. All matching contributions are immediately vested. Expense recognized by the Company for matching contributions made in accordance with the 401(k) Plan was $1.0 million and $1.2 million for the years ended December 31, 2025 and December 31, 2024, respectively.

13. Income Taxes

For the years ended December 31, 2025 and 2024, the Company did not record a current or deferred income tax expense or benefit. The following table reconciles the federal statutory income tax rate to the Company's effective income tax rate:

	Year Ended December 31,	
	2025	2024
Federal income tax rate	21.0 %	21.0 %
State income tax benefit	3.0 %	5.6 %
Permanent items	(0.9) %	(1.6) %
Change in fair value of warrant liability	(10.1) %	—
Research and development tax credits	0.4 %	4.8 %
Valuation allowance	(13.4) %	(29.8) %
Effective income tax rate	—	—

Deferred tax assets and liabilities reflect the net tax effects of net operating loss and tax credit carryforwards and temporary differences between the carrying amount of assets and liabilities for financial reporting and the amounts used for tax purposes.

Significant components of the Company's deferred tax assets and liabilities were as follows:

(in thousands)	December 31, 2025	December 31, 2024
Deferred tax assets:		
Accrued expenses	$ 737	$ 1,651
Federal net operating loss carryforwards	86,456	51,217
State net operating loss carryforwards	24,592	14,828
Tax credits	25,575	22,725
Stock compensation	1,031	1,733
R&D capitalization	36,768	46,973
Amortization	57,641	1,582
Lease liability	801	8,634
Total deferred tax assets	233,601	149,343
Valuation allowance	(232,721)	(139,216)
Net total deferred tax assets	$ 880	$ 10,127
Deferred tax liabilities:		
Lease right of use asset	(784)	(9,495)
Depreciation and amortization	(96)	(632)
Total deferred tax liabilities	$ (880)	$ (10,127)
Net deferred tax assets	$ —	$ —

The Company has weighed the positive and negative evidence to assess the recoverability of its deferred tax assets. Realization of future tax benefits is dependent on many factors, including the Company's ability to generate taxable income. After this assessment, the Company determined it was more likely than not that the Company will not realize the benefit of its deferred tax assets, net of deferred tax liabilities. As a result, the Company has provided a full valuation allowance against its net deferred tax assets. The valuation allowance for deferred tax assets as of December 31, 2025 and 2024 was $232.7 million and $139.2 million, respectively. For the years ended December 31, 2025 and 2024, the Company recorded an increase in the valuation allowance of $93.5 million and $34.8 million, respectively, primarily related to net operating losses incurred by the Company and an increase in amortization in the year ended December 31, 2025.

As of December 31, 2025, the Company had gross U.S. federal net operating loss carryforwards of $411.7 million including $409.8 million that had an indefinite carryforward period and $1.9 million that were subject to expiration at various dates through 2037. As of December 31, 2025, the Company had state net operating loss carryforwards of $389.4 million which will expire at various dates through 2045. As of December 31, 2025, the Company had U.S. federal research and development tax credit carryforwards of $19.2 million which will expire at various dates through 2045 and state research and credit carryforwards of $8.0 million which will expire at various dates through 2040. The net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities.

Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Code, respectively, 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 determined whether an ownership change has occurred and as such, the Company's net operating losses may be limited. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research development credit carryforwards before utilization.

The Company has not, as yet, conducted a study of research and development credit carryforwards. Such a study, once undertaken by the Company, may result in an adjustment to the research and development credit carryforwards. However, a full valuation allowance has been provided against the Company's research and development credits and, if any adjustment is required, such adjustment would be offset by an adjustment to the

valuation allowance. Thus, there would be no impact to the balance sheet or statement of operations if any adjustment is required.

As of December 31, 2025 and 2024, the Company did not have any unrecognized tax benefits. Any future interest and penalties related to income tax matters would be recognized in the provision for income tax. As of December 31, 2025 and 2024, the Company did not have a balance of accrued interest and penalties related to uncertain tax positions.

The Company files income tax returns in the United States and various states. As of December 31, 2025, there were no income tax examinations in progress.

The tax years 2022 through present remain open to examination by major taxing jurisdictions to which the Company is subject, which are primarily in the United States. In addition, tax years prior to 2016 resulted in losses and the Company also generated research and development tax credits during those years. Since carryforward attributes generated in these years may be utilized in future years, years prior to 2022 may still be adjusted upon examination by the Internal Revenue Service or state tax authorities if they have or will be used in a future period.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted in the U.S. The OBBBA includes significant provisions, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to the international tax framework and the restoration of favorable tax treatment for certain business provisions, most notably Section 174 capitalization of domestic research and development costs. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented through 2027. There was not a significant impact on the Company's tax expense or effective tax rate for year ended December 31, 2025 associated with the OBBBA.

14. Net Loss Per Share

The following table sets forth the computation of the Company's basic and diluted net loss per share for the years ended December 31, 2025 and 2024:

	Year Ended December 31,	
(in thousands, except share and per share amounts)	**2025**	**2024**
Numerator:		
Net loss	$ (695,981)	$ (116,914)
Denominator:		
Weighted-average number of common shares outstanding, basic and diluted	9,871,866	3,435,533
Net loss per share, basic and diluted	$ (70.50)	$ (34.03)

The Company's potentially dilutive securities were stock options, unvested restricted stock, restricted stock units, and warrants. Based on the amounts outstanding at December 31, 2025 and 2024, the Company excluded the following potential common shares from the computation of diluted net loss per share because including them would have had an anti-dilutive effect:

	As of December 31,	
	2025	**2024**
Options to purchase common stock	6,608,266	457,152
Restricted stock units	186,325	66,046
Warrants	49,412,312	3,491,953

15. Segments

The Company operates and manages its business as one reportable and operating segment, centered around the commercial development of its product candidates. The Company's chief operating decision maker ("CODM") is the Chief Executive Officer ("CEO").

The Company's CODM reviews consolidated operating results, manages the business on a consolidated basis and utilizes consolidated net loss from the consolidated statements of operations and comprehensive loss to make decisions about allocating resources and assessing performance for the entire Company. Consolidated net loss is also used to monitor budget to actual results. The CODM is additionally regularly provided with more detailed expense information at the program level.

The following table is a summary of the segment profit or loss, including significant segment expenses (in thousands):

	Year ended December 31,	
	2025	2024
Segment expenses:		
Telitacicept - gMG[(a)]	$ 24,959	—
Telitacicept - SjD[(a)]	3,300	—
Trem-cel[(a)]	9,437	18,905
VCAR33[(a)]	6,000	8,930
Other research and development[(a)]	229,092	12,608
Salaries and benefits	39,238	44,165
General corporate activities	31,540	19,856
Other segment items[(b)]	352,415	12,450
Segment expenses:	695,981	116,914
Segment net loss	$ (695,981)	$ (116,914)

[(a)] Includes only external research and development expenditures.
[(b)] Other segment items are primarily comprised of interest income on marketable securities and certain non-cash expenses such as changes in warrant liability, stock-based compensation, depreciation expense, and non-cash lease expense.

The measure of segment assets is reported on the consolidated balance sheet as total assets. The CODM additionally reviews cash, cash equivalents and marketable securities when reviewing segment assets. As of December 31, 2025, the Company's cash, cash equivalents and marketable securities were $455.2 million. The Company does not provide its CODM with any more detailed segment asset information than what is included on the Company's consolidated balance sheets. The Company's assets are located entirely in the United States.

16. Restructuring Plan

On May 5, 2025, the Company's board of directors approved the wind down of the Company's then clinical and manufacturing operations and the initiation of a process to explore strategic alternatives to maximize shareholder value (the "Restructuring Plan"). The Company publicly announced this plan on May 8, 2025.

In conjunction with the Restructuring Plan, the Company announced a reduction of its workforce by 154 full-time employees, or approximately 99% of the Company's then-current employee base. Total severance and benefit costs incurred as of December 31, 2025 were $13.1 million.

As discussed in Notes 5 and 9, in connection with the Restructuring Plan, the Company sold certain long-lived assets as well as abandoned a right-of-use asset and other property and equipment. The disposals and abandonments resulted in a loss on disposal of $3.3 million, accelerated depreciation of $1.5 million, and accelerated amortization of right-of-use assets of $11.2 million, all of which were incurred during the three months ended June 30, 2025. Management concluded that the disposals do not meet the criteria for discontinued operations as they do not represent a strategic shift that will have a major effect on the Company's operations and financial results.

Restructuring Costs

The Company incurred a total of $29.7 million of restructuring costs in the year ended December 31, 2025.

A summary of the restructuring costs recorded in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2025 is as follows:

	Year ended December 31, 2025					
(in thousands)	Severance and Benefits Costs	Stock-based Compensation	Accelerated Depreciation on Long-lived Assets	Accelerated Amortization on Right-of-use Assets	Loss on Disposal of Long-lived Assets	Total Restructuring Cost Recorded
Research and development expense	8,379	—	1,230	10,159	3,303	23,071
General and administrative expense	4,738	549	284	1,064	—	6,635
Total	$ 13,117	$ 549	$ 1,514	$ 11,223	$ 3,303	$ 29,706

Restructuring Liability

The following table provides details of the severance and benefit costs with the remaining balance of the liability recorded in accrued liabilities on the consolidated balance sheets as of December 31, 2025:

(in thousands)	Restructuring Liability
Restructuring liability as of January 1, 2025	—
Severance and benefit restructuring costs incurred	13,117
Cash Payments	(13,023)
Liability included in accrued liabilities at December 31, 2025	$ 94

17. Related Party Transactions

June 2025 Private Placement

As discussed in Note 7, on June 25, 2025, the Company entered into a purchase agreement with certain institutional investors pursuant to which the Company issued and sold warrants to purchase the Company's common stock. R.A. Capital Healthcare Fund, L.P. purchased warrants to purchase up to 10,000,000 shares of the Company's common stock for gross proceeds of $50.0 million. R.A. Capital Healthcare Fund, L.P. and its affiliates beneficially own more than 5% of the Company's common stock. Joshua Resnick, M.D., a member of the Company's board of directors at the time of the transaction, is a managing director at RA Capital Management L. P., an affiliate of R.A. Capital Healthcare Fund, L.P. On August 25, 2025, Dr. Resnick resigned as a director, and on August 27, 2025, the Company's board of directors appointed Sarah Reed to fill the vacancy created by the resignation of Dr. Resnick. Ms. Reed serves as general counsel of R.A. Capital Management, L.P. The terms of the warrants are further described in Note 7 and none of the warrants have been exercised as of December 31, 2025.

Sale of Intellectual Property

On September 22, 2025, the Company entered into an Asset Purchase Agreement pursuant to which the Company agreed to sell certain intellectual property related to the Company's previous product candidates trem-cel and VCAR33 to SyzygyMed Inc. ("Syzygy") for $1.1 million in cash consideration. Syzygy is wholly owned by Reprogrammed Interchange LLC, a beneficial owner of more than 5% of the Company's common stock.

December 2025 Private Placement

As also discussed in Note 7, on December 15, 2025, the Company entered into a purchase agreement with certain investors pursuant to which the Company issued and sold shares of the Company's common stock. R.A. Capital Healthcare Fund, L.P. purchased 4,625,346 shares of the Company's common stock for gross proceeds of $50.0 million. RA Capital Healthcare Fund, L.P. and its affiliates beneficially own more than 5% of the Company's common stock. Sarah Reed, general counsel of R.A. Capital Management, L.P., was a member of the Company's board of directors on the date of the transaction. On December 17, 2025, Ms. Reed resigned as a director, and the Company's board of directors appointed Andrew Levin, M.D., Ph.D., to fill the vacancy created by Ms. Reed's

resignation. Mr. Levin serves as a Partner at R.A. Capital Management, L.P. Additionally, as part of the purchase agreement entered into on December 15, 2025, an entity affiliated with Forbion Growth Opportunities Fund ("Forbion") purchased 3,237,742 shares of the Company's common stock for gross proceeds of $35.0 million. Forbion became a greater than 5% stockholder of the Company's outstanding shares in connection with this purchase. In conjunction with Forbion's purchase, the Company's board of directors appointed as a new board member Wouter Joustra, a General Partner of Forbion.

18. Subsequent Events

March 2026 Private Placement

On March 26, 2026, the Company entered into a securities purchase agreement with certain institutional investors pursuant to which the Company, in a private placement, agreed to issue and sell an aggregate of 5,338,078 shares of common stock, at a price per share of $14.05, for gross proceeds of $75.0 million before deducting offering expenses. The closing of the private placement is expected to occur on March 30, 2026, subject to satisfaction of customary closing conditions.