UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 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

N/A

(Former name or former address, if changed since last report)

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

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

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

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

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

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

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

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

Number of shares of the registrant's Common Stock outstanding as of November 7, 2025 was 10,410,630.

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Note Regarding Company References

Throughout this Quarterly Report on Form 10-Q, 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

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, 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," "expect," "plan," "anticipate," "could," "might," "intend," "target," "ongoing," "project," "estimate," "believe," "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 Quarterly Report on Form 10-Q 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, 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 preclinical 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 competitive position and the development of and projections relating to our competition or our industry;
- the impact of laws and regulations; and
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q are made as of the date of this Quarterly Report on

Form 10-Q, 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. You should refer to the "Risk Factors" section in this Quarterly Report and the "Summary Risk Factors" and "Risk Factors" sections in our Annual Report on Form 10-K for the year ended December 31, 2024 for a discussion of material factors that could cause actual results or events to differ materially from the forward-looking statements that we make.

This Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q 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 Quarterly 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 our 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 have contracted and 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 reliance on third parties increases the risk that we will not have sufficient quantities and quality of such materials, product candidates or any products that we may develop and commercialize, 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—FINANCIAL INFORMATION

Item 1. Financial Statements (Unaudited).

VOR BIOPHARMA INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(UNAUDITED)

(in thousands, except share and per share amounts)	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 160,462	$ 81,949
Marketable securities	10,000	9,977
Prepaid expenses	747	4,201
Other current assets	1,277	380
Total current assets	172,486	96,507
Restricted cash	168	2,413
Property and equipment, net	271	6,581
Operating lease right-of-use assets	3,043	35,007
Other assets	269	2,383
Total assets	$ 176,237	$ 142,891
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 885	$ 1,505
Accrued liabilities	17,702	12,892
Operating lease liabilities	252	4,215
Total current liabilities	18,839	18,612
Non-current liabilities:		
Operating lease liabilities, less current portion	2,807	27,615
Warrant liabilities	2,380,078	—
Total liabilities	2,401,724	46,227
Stockholders' equity (deficit)[1]:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized as of September 30, 2025 and December 31, 2024; 0 shares issued and outstanding as of September 30, 2025 and December 31, 2024	—	—
Common stock, $0.0001 par value; 800,000,000 and 400,000,000 shares authorized as of September 30, 2025 and December 31, 2024, respectively; 9,026,922 and 6,238,799 shares issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	15	13
Additional paid-in capital	650,319	553,623
Accumulated other comprehensive income	12	22
Accumulated deficit	(2,875,833)	(456,994)
Total stockholders' (deficit) equity	(2,225,487)	96,664
Total liabilities and stockholders' equity	$ 176,237	$ 142,891

[1] All share and per share amounts have been restated for all periods presented on a retroactive basis to reflect a one-for-twenty reverse stock split effected in September 2025. See Note 2 for details.

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

VOR BIOPHARMA INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(UNAUDITED)

(in thousands, except share and per share amounts)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 14,142	$ 21,817	$ 302,342	$ 67,962
General and administrative	13,965	6,696	33,340	21,912
Total operating expenses	$ 28,107	$ 28,513	$ 335,682	$ 89,874
Loss from operations	(28,107)	(28,513)	(335,682)	(89,874)
Other income (expense):				
Interest income	1,731	954	3,073	3,672
Other income	4,149	—	4,149	—
Change in fair value of warrant liabilities	(790,457)	—	(2,090,379)	—
Total other (expense) income	(784,577)	954	(2,083,157)	3,672
Net loss	$ (812,684)	$ (27,559)	$ (2,418,839)	$ (86,202)
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (121.63)	$ (8.05)	$ (378.11)	$ (25.25)
Weighted-average common shares outstanding, basic and diluted[1]	6,681,794	3,423,499	6,397,121	3,413,625
Other comprehensive income (loss):				
Unrealized (loss) gain on available for sale marketable securities	(2)	138	(10)	145
Total other comprehensive (loss) income:	(2)	138	(10)	145
Comprehensive loss	$ (812,686)	$ (27,421)	$ (2,418,849)	$ (86,057)

[1] All share and per share amounts have been restated for all periods presented on a retroactive basis to reflect a one-for-twenty reverse stock split effected in September 2025. See Note 2 for details.

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

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

(in thousands, except share amounts)	Common Stock[1] Shares	Common Stock[1] Amount	Additional Paid-In Capital	Accumulated other comprehensive income	Accumulated Deficit	Total Stockholders' Equity (Deficit)
Balance at 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	3,769	—	(55)	—	—	(55)
Issuance costs for private placement	—	—	12	—	—	12
Stock-based compensation expense	—	—	1,933	—	—	1,933
Other comprehensive income	—	—	—	1	—	1
Net loss	—	—	—	—	(32,486)	(32,486)
Balance at March 31, 2025	6,242,568	$ 13	$ 555,513	$ 23	$ (489,480)	$ 66,069
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	39,721	—	65	—	—	65
Stock-based compensation expense	—	—	1,844	—	—	1,844
Other comprehensive income	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	(1,573,669)	(1,573,669)
Balance at June 30, 2025	6,282,289	$ 13	$ 557,422	$ 14	$ (2,063,149)	$ (1,505,700)
Issuance of common stock upon vesting of RSUs, net of shares withheld for taxes, and exercise of stock options	58,210	—	1,935	—	—	1,935
Issuance of common stock from at-the-market sales agreement	540,750	1	20,256	—	—	20,257
Issuance of common stock from exercise of warrants	2,145,673	1	62,677	—	—	62,678
Stock-based compensation expense	—	—	8,029	—	—	8,029
Other comprehensive loss	—	—	—	(2)	—	(2)
Net loss	—	—	—	—	(812,684)	(812,684)
Balance at September 30, 2025	9,026,922	$ 15	$ 650,319	$ 12	$ (2,875,833)	$ (2,225,487)

(in thousands, except share amounts)	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated other comprehensive loss (income)	Accumulated Deficit	Total Stockholders' Equity
Balance at 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, and exercise of stock options	9,121	—	(169)	—	—	(169)
Issuance of common stock from at-the-market sales agreement	6,973	—	213	—	—	213
Stock-based compensation expense	—	—	3,081	—	—	3,081
Other comprehensive loss	—	—	—	(10)	—	(10)
Net loss	—	—	—	—	(30,804)	(30,804)
Balance at March 31, 2024	3,411,166	$ 7	$ 493,999	$ (87)	$ (370,884)	$ 123,035
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	6,977	—	74	—	—	74
Stock-based compensation expense	—	—	2,793	—	—	2,793
Other comprehensive income	—	—	—	17	—	17
Net loss	—	—	—	—	(27,839)	(27,839)
Balance at June 30, 2024	3,418,143	$ 7	$ 496,866	$ (70)	$ (398,723)	$ 98,080
Issuance of common stock upon vesting of RSUs, net of shares withheld for taxes, and exercise of stock options	9,649	—	(57)	—	—	(57)
Issuance of common stock from at-the-market sales agreement	5,481	—	89	—	—	89
Stock-based compensation expense	—	—	2,264	—	—	2,264
Other comprehensive income	—	—	—	138	—	138
Net loss	—	—	—	—	(27,559)	(27,559)
Balance at September 30, 2024	3,433,273	$ 7	$ 499,162	$ 68	$ (426,282)	$ 72,955

[1] All share and per share amounts have been restated for all periods presented on a retroactive basis to reflect a one-for-twenty reverse stock split effected in September 2025. See Note 2 for details.

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

VOR BIOPHARMA INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(UNAUDITED)

(in thousands)	Nine Months Ended September 30, 2025	2024
Cash flows from operating activities		
Net loss	$ (2,418,839)	$ (86,202)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	2,864	2,663
Non-cash lease expense	5,540	3,752
Stock-based compensation	11,806	8,138
Transaction costs for issuance of pre-funded warrants	643	—
Change in fair value of warrant liabilities	2,090,379	—
Interest amortization on marketable securities	(33)	(1,137)
Loss on sale of property and equipment	3,303	—
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	(2,347)	(2,806)
Prepaid expenses and other current assets	2,557	(859)
Accounts payable, accrued liabilities and other current liabilities	4,724	(1,446)
Other assets	2,114	2,300
Net cash used in operating activities	(119,912)	(75,597)
Cash flows from investing activities		
Purchases of marketable securities	—	(9,914)
Proceeds from maturities of marketable securities	—	107,000
Purchases of property and equipment	(627)	(157)
Proceeds from sales of property and equipment	799	—
Net cash provided by investing activities	172	96,929
Cash flows from financing activities		
Payment of issuance costs related to private placement and pre-funded warrants	(1,194)	—
Proceeds from the issuance of pre-funded warrants	175,000	—
Proceeds from the issuance of common stock from at-the-market sales agreement, net of issuance costs	20,257	297
Repurchases of shares for tax withholdings upon vesting of restricted stock unit awards	(106)	(303)
Proceeds from stock option exercises and the issuance of shares under ESPP	2,051	112
Net cash provided by financing activities	196,008	106
Net increase in cash, cash equivalents and restricted cash equivalents	76,268	21,438
Cash, cash equivalents and restricted cash equivalents, beginning of period	$ 84,362	$ 33,773
Cash, cash equivalents and restricted cash equivalents, end of period	$ 160,630	$ 55,211
Supplemental disclosure of non-cash activities		
Right-of-use assets obtained in exchange for lease obligations	$ 3,079	$ —
Purchases of property and equipment in accounts payable and accrued liabilities	$ 29	$ —
Unrealized (loss) gain on available-for-sale securities	$ (10)	$ 145
Financing costs associated with the sale of common stock included in accounts payable and accrued expenses	$ —	$ 22

A reconciliation of the cash, cash equivalents and restricted cash equivalents reported within the condensed 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 Nine Months Ended September 30, 2025	2024
Cash and cash equivalents	$ 160,462	$ 52,798
Restricted cash equivalents	168	2,413
Total cash, cash equivalents and restricted cash equivalents as shown on the statements of cash flows	$ 160,630	$ 55,211

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

VOR BIOPHARMA INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(UNAUDITED)

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

The Company expects that its existing cash, cash equivalents and marketable securities as of September 30, 2025 of $170.5 million, in addition to the $49.8 million in net proceeds from at-the-market sales during October 2025 and the approximately $93.7 million in net proceeds from the sale of common stock in an underwritten public offering that closed on November 12, 2025, 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.

As of September 30, 2025, the Company has an accumulated deficit of $2,875.8 million. 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

The accompanying condensed 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") and Accounting Standards Updates ("ASU") issued by the Financial Accounting Standards Board ("FASB").

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 authorized shares of the 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 condensed 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 condensed consolidated financial statements in conformity with GAAP requires the Company's management to make estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the condensed 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 condensed 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, stock-based compensation expense, the fair value of financial instruments including warrants, and research and development expenses.

Unaudited Interim Financial Information

The condensed consolidated financial statements of the Company included herein have been prepared pursuant to the rules and regulations of the Securities and Exchange Commission (the "SEC"). The unaudited condensed consolidated financial statements include the accounts of the Company and its wholly owned subsidiary. All intercompany transactions and balances have been eliminated in consolidation. In the opinion of management, the information furnished reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair presentation of the results for the reported interim periods. The results of operations for interim periods are not necessarily indicative of results to be expected for the full year or any other interim period.

The accompanying condensed consolidated balance sheet as of December 31, 2024 has been derived from the Company's audited consolidated financial statements for the year ended December 31, 2024. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to GAAP or the rules and regulations of the SEC. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These unaudited interim condensed consolidated financial statements should be read in conjunction with the audited annual consolidated financial statements and related notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2024 (the "2024 Annual Report").

Other than the policies listed below, there have been no material changes to the Company's significant accounting policies as described in the 2024 Annual Report.

Warrants

The Company accounts for warrants to purchase its common stock 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 the 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 remain outstanding.

Warrants classified as equity are recorded at fair value as of the date of issuance on the Company's condensed consolidated balance sheets and no further adjustments to their initial valuation will be subsequently made. Warrants classified as liabilities are recorded on the Company's condensed 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) on the condensed consolidated statement of operations and comprehensive loss.

As of September 30, 2025, the Company has both equity-classified and liability-classified warrants outstanding.

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. See Note 12 for additional information on the severance expense that the Company recognized for employees terminated in connection with the Restructuring Plan (as defined below).

3. Marketable Securities

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

(in thousands)	September 30, 2025			
	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Maturing in one year or less				
U.S. Treasuries	$ 9,988	$ 12	$ —	$ 10,000
Total	$ 9,988	$ 12	$ —	$ 10,000

(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 either September 30, 2025 or December 31, 2024. Additionally, the Company did not record any impairments to marketable securities or reserves for credit losses related to its marketable debt securities during the periods presented.

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)	September 30, 2025			
	Level 1	Level 2	Level 3	Total
Cash equivalents				
Money market funds	$ 160,212	$ —	$ —	$ 160,212
Marketable securities				
U.S. Treasuries	—	10,000	—	10,000
Restricted cash equivalents				
Money market funds	168	—	—	168
Liabilities				
Warrant liabilities	—	2,380,078	—	2,380,078

(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
Restricted cash equivalents				
Money market funds	2,413	—	—	2,413

The fair value of the Company's cash equivalents and restricted cash equivalents is determined based on quoted market prices in active markets with no valuation adjustment. The fair value of marketable securities and warrant liabilities is determined based on observable market inputs. There were no transfers between levels during the nine months ended September 30, 2025.

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)	September 30, 2025	December 31, 2024
Laboratory equipment	$ —	$ 9,625
Manufacturing equipment	—	7,082
Computer equipment	—	446
Furniture, fixtures and other	272	606
Construction in progress	—	36
Total	272	17,795
Less: Accumulated depreciation	(1)	(11,214)
Property and equipment, net	$ 271	$ 6,581

Depreciation expense for the nine months ended September 30, 2025 was $2.9 million. An immaterial amount of depreciation was recognized during the three months ended September 30, 2025. Depreciation expense for the three and nine months ended September 30, 2024 was $0.9 million and $2.7 million, respectively.

In connection with the Restructuring Plan, during the nine months ended September 30, 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 condensed consolidated statements of operations and comprehensive loss. Additionally, the Company abandoned certain long-lived assets, resulting in the acceleration of depreciation expense in the amount of approximately $1.5 million during the nine months ended September 30, 2025, of which approximately $1.2 million and $0.3 million was recorded as research and development expense and general and administrative expense, respectively, in the condensed consolidated statements of operations and comprehensive loss.

6. Accrued Liabilities

Accrued liabilities consisted of the following:

(in thousands)	September 30, 2025	December 31, 2024
Employee-related expenses, including Restructuring Liability (Note 12)	$ 3,026	$ 5,852
Professional fees	1,609	1,461
Clinical expenses	5,478	3,835
Manufacturing expenses	7,361	516
Research and development expenses	35	872
Other	193	356
Total accrued liabilities	$ 17,702	$ 12,892

7. Stockholders' Equity and Warrants

2024 Private Placement

On December 27, 2024, the Company entered into a purchase agreement with certain institutional investors (collectively, the “2024 Purchasers”), pursuant to which the Company issued and sold to the 2024 Purchasers 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 seven years from issuance 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.

Accordingly, the 2024 Warrants are classified as equity and accounted for as a component of additional paid-in capital at the time issued.

As of September 30, 2025, none of the 2024 Warrants have been exercised.

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 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 ("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. On August 25, 2025, the stockholders approved the issuance of the underlying shares and on September 17, 2025, the Company filed a Certificate of Amendment to its Amended and Restated Certificate of Incorporation with the Secretary of State of the State of Delaware to effect a reverse stock split, resulting in sufficient available authorized shares for warrant holders to exercise 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 condensed consolidated statements of operations and comprehensive loss. The valuation of the 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 also determined that both the 2024 Warrants and 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 three and nine months ended September 30, 2025 as the effect would be antidilutive.

During the three and nine months ended September 30, 2025, 2,145,829 of the 2025 Warrants were exercised for an immaterial amount of net proceeds. The remaining 48,854,170 outstanding 2025 Warrants have a fair value of $2,380.1 million as of September 30, 2025.

8. Stock-Based Compensation

2023 Inducement Plan

As of September 30, 2025, the Company had 1,893,219 shares of its common stock available for future issuance under the 2023 Inducement Plan.

Amended and Restated 2021 Equity Incentive Plan

As of September 30, 2025, the Company had 1,739,508 shares of its common stock available for future issuance under its Amended and Restated 2021 Equity Incentive Plan.

Stock Options

The Company's stock options generally vest ratably over a four-year period and have a contractual term of ten years. The weighted-average assumptions used principally in determining the fair value of new options granted during the periods presented were as follows:

	Nine Months Ended September 30,	
	2025	2024
Expected term (in years)	6.1	6.0
Expected volatility	98.1%	88.9%
Risk-free interest rate	3.9%	4.1%
Dividend yield	—	—

During the nine months ended September 30, 2025 and 2024, the Company granted new stock options to purchase 5,150,913 shares and 108,631 shares of its common stock, respectively, with a weighted-average grant-date fair value of $17.30 and $29.80 per share, respectively. As of September 30, 2025, total unrecognized compensation expense related to stock options was $82.3 million, which is expected to be recognized over a weighted-average period of 2.1 years. The intrinsic value of stock options exercised during the nine months ended September 30, 2025 was $0.8 million.

Option Repricing

On February 3, 2025, the Company's board of directors approved a stock option repricing (the "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 (the "Retention Period"). If any such repriced option is exercised prior to the end of the Retention Period, 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 Option Repricing represents a modification of share-based awards and calculated incremental compensation cost of approximately $1.9 million resulting from the modification. However, as the conditions of the modified terms were not expected to be met as of June 30, 2025, the Company reversed any previously recognized incremental compensation cost associated with the modification during the quarter ended June 30, 2025. As of September 30, 2025, the Company still expects that the conditions of the modified terms will not be met.

Restricted Stock Units

During the nine months ended September 30, 2025 and 2024, the Company granted 919,133 restricted stock units and 59,723 restricted stock units, respectively, with a weighted-average grant date fair value of $47.67 and $45.81 per share, respectively. As of September 30, 2025, total unrecognized compensation expense related to restricted stock units was $40.1 million, which is expected to be recognized over a weighted-average period of 2.2 years.

Employee Stock Purchase Plan

As of September 30, 2025, the Company had 115,932 shares of its common stock available for issuance under its Employee Stock Purchase Plan ("ESPP"). During the nine months ended September 30, 2025, the Company issued 33,477 shares with a weighted-average purchase price of $2.91 under the ESPP, which resulted in an immaterial amount of compensation expense. During the nine months ended September 30, 2024, the Company issued 3,706 shares with a weighted-average purchase price of $29.58 under the ESPP, which resulted in an immaterial amount of compensation expense.

Equity Award Modification

In connection with the Restructuring Plan, the Company executed an equity award modification which accelerated the vesting of the former Chief Executive Officer's restricted stock units and options as a part of his consulting and separation agreement. The modification additionally extended the period for which vested options can be exercised following the employee's final day of employment. During the nine months ended September 30, 2025, the Company recognized $0.5 million of stock-based compensation expense related to the equity award modification as general and administrative expense within the condensed consolidated statement of operations and comprehensive loss.

Stock-Based Compensation

Stock-based compensation expense was allocated as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	**2025**	**2024**	**2025**	**2024**
Research and development	$ 491	$ 1,148	$ 2,132	$ 3,825
General and administrative	7,538	1,116	9,674	4,313
Total stock-based compensation expense	$ 8,029	$ 2,264	$ 11,806	$ 8,138

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 made 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 has accelerated amortization of the right-of-use asset and de-recognized any remaining balances during the quarter ended June 30, 2025.

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 condensed consolidated balance sheets.

The elements of lease expense were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	2025	2024	2025	2024
Operating lease cost	$ 83	$ 1,951	$ 15,119	$ 5,853
Variable lease cost	57	681	1,542	1,890
Total lease cost	$ 140	$ 2,632	$ 16,661	$ 7,743

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

(in thousands except weighted-average amounts)	September 30, 2025	December 31, 2024
Assets		
Operating lease right-of-use assets	$ 3,043	$ 35,007
Liabilities		
Operating lease liabilities, current	$ 252	$ 4,215
Operating lease liabilities, non-current	2,807	27,615
Total lease liabilities	$ 3,059	$ 31,830
Weighted-Average Lease Term and Discount Rate		
Weighted-average remaining lease term (years)	5.9	5.7
Weighted-average discount rate	6.7%	8.2%

The following table represents other lease activity:

(in thousands)	Nine Months Ended September 30, 2025	2024
Other Information		
Cash paid for amounts included in the measurement of lease liabilities		
Operating cash flows for operating leases	$ 11,920	$ 4,906

10. Significant Agreements

Telitacicept License Agreement

On June 25, 2025, the Company and RemeGen Co., Ltd. ("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 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 were 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 nine months ended September 30, 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 condensed consolidated statement of operations and comprehensive loss during the nine months ended September 30, 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 condensed consolidated statement of cash flows.

Pursuant to the terms of the Telitacicept License Agreement, 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 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 September 30, 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.

In connection with the aforementioned sales, the Company assigned certain license agreements 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 as discussed within the 2024 Annual Report.

11. Net Loss Per Share

The following table sets forth the computation of the Company's basic and diluted net loss per share for the three and nine months ended September 30, 2025 and 2024:

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands, except share and per share amounts)	2025	2024	2025	2024
Numerator:				
Net loss attributable to common stockholders	$ (812,684)	$ (27,559)	$ (2,418,839)	$ (86,202)
Denominator:				
Weighted-average number of common shares outstanding, basic and diluted	6,681,794	3,423,499	6,397,121	3,413,625
Net loss per share attributable to common stockholders, basic and diluted	$ (121.63)	$ (8.05)	$ (378.11)	$ (25.25)

The Company's potentially dilutive securities were stock options, unvested restricted stock, restricted stock units, and warrants. Based on the amounts outstanding as of September 30, 2025 and 2024, the Company excluded the following potential common shares from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect:

	As of September 30,	
	2025	2024
Options to purchase common stock	5,280,272	485,234
Unvested restricted stock	—	128
Restricted stock units	883,136	70,847
Warrants	52,346,123	—

12. 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 September 30, 2025 were $13.1 million. These costs were recognized in accordance with either ASC 712 or ASC 420, depending on the agreements with impacted employees.

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 nine months ended September 30, 2025. During the three months ended September 30, 2025, the Company revised the total estimated severance and benefit costs incurred by $1.2 million as actual payments made to or on behalf of terminated employees differed from estimated costs, partially due to changes in benefits elections and coverage.

A summary of the restructuring costs recorded in the condensed consolidated statements of operations and comprehensive loss for both the three and nine months ended September 30, 2025 is as follows:

(in thousands)	Three Months Ended September 30, 2025					
	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	$ (578)	$ —	$ —	$ —	$ —	$ (578)
General and administrative expense	(451)	—	—	—	—	(451)
Total restructuring costs	$ (1,029)	$ —	$ —	$ —	$ —	$ (1,029)

(in thousands)	Nine Months Ended September 30, 2025					
	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 restructuring costs	$ 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 condensed consolidated balance sheets as of September 30, 2025:

(in thousands)	Restructuring Liability
Restructuring liability as of June 30, 2025	$ 11,514
Additions	181
Cash payments	(8,296)
Adjustments	(1,210)
Liability included in accrued liabilities at September 30, 2025	$ 2,189

13. 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"). On June 25, 2025, the Company appointed a new CEO who has taken over the role of CODM from the previous CEO.

The Company's CODM reviews consolidated operating results, manages the business on a consolidated basis and utilizes consolidated net loss from the condensed 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. Beginning during the quarter ended September 30, 2025, such programs also included "Telitacicept - MG", representing the Company's external spend on the research and development of a new product candidate, acquired through the Telitacicept License Agreement as described in Note 10.

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

(in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Segment expenses:				
Telitacicept - gMG[(a)]	$ 13,069	$ —	$ 13,069	$ —
Trem-cel[(a)]	(102)	4,068	9,375	12,548
VCAR33[(a)]	(60)	1,778	5,995	6,140
Other research and development[(a)]	456	3,080	228,595	9,374
Salaries and benefits	2,599	10,742	32,075	33,259
General corporate activities	4,093	4,924	25,956	15,170
Other segment items[(b)]	792,629	2,967	2,103,774	9,711
Segment expenses:	812,684	27,559	2,418,839	86,202
Segment net loss	$ (812,684)	$ (27,559)	$ (2,418,839)	$ (86,202)

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

The measure of segment assets is reported on the condensed consolidated balance sheets as total assets. The CODM additionally reviews cash, cash equivalents and marketable securities when reviewing segment assets. As of September 30, 2025, the Company's cash, cash equivalents and marketable securities were $170.5 million. The Company does not provide its CODM with any more detailed segment asset information than what is included on the Company's condensed consolidated balance sheets.

14. Related Party Transactions

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. RA 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. RA 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 RA 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 RA 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 September 30, 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 five percent of the Company's common stock.

15. Subsequent Events

At-the-Market Sales Activity

During October 2025, the Company sold 1,370,111 shares of common stock under the existing at-the-market sales agreement with Stifel, Nicolaus & Company, Incorporated as the agent (the "Stifel ATM Facility"). The shares were sold at a weighted-average price per share of $36.73 for aggregate net proceeds of $49.8 million, after deducting commissions. Following these sales, $48.9 million remained available to be sold under the Stifel ATM Facility.

Underwritten Public Offering

On November 12, 2025, the Company issued and sold 10,000,000 shares of common stock in an underwritten public offering at a price to the public of $10.00 per share, for net proceeds of approximately $93.7 million, after deducting the underwriting discounts and commissions and estimated offering expenses. In addition, on November 12, 2025, the underwriters exercised in full their option to purchase 1,500,000 additional shares of common stock at the public offering price, less the underwriting discounts and commissions. The closing of the purchase of the additional shares is expected to occur on November 14, 2025, subject to satisfaction of customary closing conditions, for additional expected net proceeds of $14.1 million, after deducting the underwriting discounts and commissions.

Item 2. 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 the unaudited condensed consolidated financial statements and related notes included elsewhere in this Quarterly Report on Form 10-Q and with the consolidated financial statements and related notes included in our Annual Report on Form 10-K for the year ended December 31, 2024 (the "2024 Annual Report"). Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly 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" in our 2024 Annual Report and in other reports we have filed or may file with the SEC, 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"). Telitacicept is approved in China for the treatment of generalized myasthenia gravis ("gMG"), systemic lupus erythematosus ("SLE") and rheumatoid arthritis ("RA"). RemeGen has also filed Biologics License Applications ("BLAs") in China for the treatment of Sjögren's disease ("SD") and IgA nephropathy ("IgAN").

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.

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 currently being evaluated in a global Phase 3 clinical trial, for which we are assuming 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.

The data from the open-label treatment period (Part B) of the Phase 3 trial were presented at the American Association of Neuromuscular & Electrodiagnostic Medicine ("AANEM") 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 from baseline, 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, SD 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.

Our broader development plan includes exploring development of telitacicept in additional autoantibody-driven diseases where BAFF / APRIL signaling is a validated target, supported by prior clinical experience and mechanistic rationale. This includes Sjögren's disease, one of the most common systemic autoimmune diseases globally. There are no approved disease-modifying systemic therapies for the treatment of Sjögren's disease.

The data from the RemeGen-sponsored Phase 3 trial conducted in China evaluating telitacicept in SD were presented at the American College of Rheumatology ("ACR") Convergence meeting in October 2025. The 48-week results demonstrated:

- Mean change in ESSDAI score from baseline: 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). These data demonstrate durable, dose-dependent improvement in systemic disease activity.

- Mean change in ESSPRI score from baseline: 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). These data showed sustained symptomatic benefit in dryness, fatigue, and pain.
- ≥3-point ESSDAI score 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 score <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 score 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 and telitacicept 80mg each produced a statistically significant 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.

Restructuring Plan

On May 5, 2025, the Company's board of directors approved the wind down of the Company's existing clinical and manufacturing operations focused on previous product candidates (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.

During the nine months ended September 30, 2025, we incurred restructuring costs of $29.7 million which is 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.

We have incurred significant operating losses since inception, including net losses of $812.7 million and $2,418.8 million for the three and nine months ended September 30, 2025, respectively and $116.9 million for the year ended December 31, 2024. As of September 30, 2025, we had an accumulated deficit of $2,875.8 million.

As of September 30, 2025, we had cash, cash equivalents and marketable securities of $170.5 million. Based on our current operating plan and considering the net proceeds of $49.8 million from the sale of shares of common stock under our at-the-market sales facility during October 2025 and the approximately $107.8 million in net proceeds and expected net proceeds from the November 2025 Offering (as defined below), we expect that our cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2027.

Critical Accounting Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our condensed consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles. The preparation of our condensed consolidated financial statements requires us to make judgments and estimates that affect the reported amounts of assets, liabilities, costs, and expenses, and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events, and various other factors that we believe to be reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions. On an ongoing basis, we evaluate our judgments and estimates in light of changes in circumstances, facts, and experience. The effects of material revisions in estimates, if any, will be reflected in the condensed consolidated financial statements prospectively from the date of change in estimates. During the nine months ended September 30, 2025, in connection with our decision to wind down clinical and manufacturing operations related to previous product candidates, we have revised certain estimates related to accrued research and development. There have been no other material changes to our critical accounting estimates from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our 2024 Annual Report.

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 current or future 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; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent, insurance, and other internal operating costs, and internal manufacturing expenses.

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 condensed 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 candidate continues through the 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 candidate or any future product candidates, if approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of our product candidate or any future 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 candidate or any future product candidate.

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.

We expect that our general and administrative expenses will increase as our business expands and we hire additional personnel to support the development of telitacicept.

Other Income (Expense), net

Interest Income

Interest income consists of interest income earned on our cash, cash equivalents, restricted 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 the Company's 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 the Company's common stock underlying the warrants.

Results of Operations

Comparison of Three and Nine Months Ended September 30, 2025 and 2024

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

	Three Months Ended September 30,		
	2025	2024	Change
Operating expenses:			
Research and development	$ 14,142	$ 21,817	$ (7,675)
General and administrative	13,965	6,696	7,269
Total operating expenses	28,107	28,513	(406)
Loss from operations	(28,107)	(28,513)	406
Other income (expense):			
Interest income	1,731	954	777
Other income	4,149	—	4,149
Change in fair value of warrant liabilities	(790,457)	—	(790,457)
Total other (expense) income	(784,577)	954	(785,531)
Net loss	$ (812,684)	$ (27,559)	$ (785,125)

	Nine Months Ended September 30,		
	2025	2024	Change
Operating expenses:			
Research and development	$ 302,342	$ 67,962	$ 234,380
General and administrative	33,340	21,912	11,428
Total operating expenses	335,682	89,874	245,808
Loss from operations	(335,682)	(89,874)	(245,808)
Other income (expense):			
Interest income	3,073	3,672	(599)
Other income	4,149	—	4,149
Change in fair value of warrant liabilities	(2,090,379)	—	(2,090,379)
Total other (expense) income	(2,083,157)	3,672	(2,086,829)
Net loss	$ (2,418,839)	$ (86,202)	$ (2,332,637)

Research and Development Expenses

The following table summarizes our research and development expenses incurred for the periods indicated (amounts in thousands):

	Three Months Ended September 30,		
	2025	2024	Change
External research expenses:			
Telitacicept - gMG	$ 13,069	$ —	$ 13,069
Trem-cel	(102)	4,068	(4,170)
VCAR33	(60)	1,778	(1,838)
Other research and development	456	3,080	(2,624)
Internal research expenses:			
Salaries and benefits (including stock-based compensation)	677	9,400	(8,723)
Manufacturing, facilities, and other research expenses	102	3,491	(3,389)
Total research and development expenses	$ 14,142	$ 21,817	$ (7,675)

	Nine Months Ended September 30,		
	2025	2024	Change
External research expenses:			
Telitacicept - gMG	$ 13,069	$ —	$ 13,069
Trem-cel	9,375	12,548	(3,173)
VCAR33	5,995	6,140	(145)
Other research and development	228,595	9,374	219,221
Internal research expenses:			
Salaries and benefits (including stock-based compensation)	23,117	28,973	(5,856)
Manufacturing, facilities, and other research expenses	22,191	10,927	11,264
Total research and development expenses	$ 302,342	$ 67,962	$ 234,380

Research and development expenses were $14.1 million for the three months ended September 30, 2025, compared to $21.8 million for the three months ended September 30, 2024. The decrease of $7.7 million was primarily due to lower stock-based compensation and personnel costs as the Company had lower headcount following the implementation of the Restructuring Plan and reduced spend on its previous programs, trem-cel and VCAR33, partially offset by the $13.1 million increase in spend on telitacicept - gMG, as the Company began research and development activities for the new program.

Research and development expenses were $302.3 million for the nine months ended September 30, 2025, compared to $68.0 million for the nine months ended September 30, 2024. The increase of $234.4 million was primarily due to the upfront consideration transferred for the license agreement with RemeGen for telitacicept (the "Telitacicept License Agreement") of $222.6 million, $13.1 million in research and development activities on telitacicept-MG as the Company began research and development activities for the new program, $3.3 million in loss on disposal of long-lived assets, and $11.2 million in accelerated depreciation and amortization due to the abandonment of long-lived assets (including the right-of-use asset) and the early termination penalty for the lease, partially offset by lower stock-based compensation and personnel costs as the Company had lower headcount following the Restructuring Plan.

General and Administrative Expenses

General and administrative expenses were $14.0 million for the three months ended September 30, 2025, compared to $6.7 million for the three months ended September 30, 2024. The increase of $7.3 million was primarily due to an increase in stock-based compensation expense.

General and administrative expenses were $33.3 million for the nine months ended September 30, 2025, compared to $21.9 million for the nine months ended September 30, 2024. The increase of $11.4 million was primarily due to an increase in stock-based compensation expense, an increase in personnel costs from the termination payments owed to employees subject to the Restructuring Plan, and an increase in facilities and other expenses from accelerated depreciation and loss on disposals of long-lived assets.

Other Income (Expense)

Other income (expense) decreased by $785.5 million during the three months ended September 30, 2025, compared to the three months ended September 30, 2024. Other income (expense) decreased by $2,086.8 million during the nine months ended September 30, 2025, compared to the nine months ended September 30, 2024. The decrease in other income (expense) in both periods was primarily due to the issuance of liability-classified warrants and the subsequent changes in fair value of the warrant liabilities resulting from changes in the quoted price of the Company's common stock underlying the warrants, offset in part by the proceeds from the sale of intellectual property related to the Company's previous programs.

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 financing transactions of approximately $711.7 million as of September 30, 2025.

On March 20, 2025, we filed a universal shelf registration statement (the "Shelf Registration Statement"), 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 September 30, 2025, $329.5 million remains available under this Shelf Registration Statement, including $99.2 million reserved for at-the-market offerings discussed below. Following the additional at-the-market sales during October 2025 and the November 2025 Offering, assuming the closing of the purchase of the 1,500,000 additional shares by the underwriters occurs, $158.9 million remains available under this Shelf Registration Statement, with $48.9 million reserved for at-the-market offerings.

At-the-Market Sales Agreement

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 will pay Stifel a commission of up to 3.0% of the gross proceeds of any common stock sold through Stifel. We sold 540,750 shares of our common stock under the Stifel ATM Facility during the nine months ended September 30, 2025 at a weighted-average price per share of $37.89 for aggregate net proceeds of $20.3 million, after deducting commissions. As of September 30, 2025, $99.2 million remained available to be sold under the Stifel ATM Facility.

During October 2025, we sold an additional 1,370,111 shares of our common stock under the Stifel ATM Facility at a weighted-average price per share of $36.73 for aggregate net proceeds of $49.8 million, after deducting commissions. Following these sales, $48.9 million remained available to be sold under the Stifel ATM Facility.

Underwritten Public Offering

On November 12, 2025, we issued and sold 10,000,000 shares of common stock in an underwritten public offering at a price to the public of $10.00 per share, for net proceeds of approximately $93.7 million, after deducting the underwriting discounts and commissions and estimated offering expenses. In addition, on November 12, 2025, the underwriters exercised in full their option to purchase 1,500,000 additional shares of common stock at the public offering price, less the underwriting discounts and commissions. The closing of the purchase of the additional shares is expected to occur on November 14, 2025, subject to satisfaction of customary closing conditions, for additional expected net proceeds of $14.1 million, after deducting the underwriting discounts and commissions (collectively, the “November 2025 Offering”).

Cash Requirements

As of September 30, 2025, we had cash, cash equivalents and marketable securities of $170.5 million. We will need to raise additional capital in the future to fund our planned future operations. However, we cannot guarantee that we will be able to obtain sufficient additional funding or that if we do obtain additional funding, that such funding will be obtainable on terms satisfactory to us. In the event that we are unable to obtain sufficient additional funding, there can be no assurance that we will be able to continue as a going concern.

Based on our current operating plan and considering the additional at-the-market sales during October 2025 and the net proceeds and expected net proceeds from the November 2025 Offering, we expect that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2027. 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 research and clinical development of our product candidate and any future product candidate, including in particular the expenses associated with our clinical trials;
- incur both internal and third party manufacturing costs to support our clinical trials of our product candidate and any future product candidate 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;
- acquire and expand office facilities; and
- experience any delays or encounter any issues with any of the above.

In addition, we expect to continue to incur 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 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 may 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 platform technology, 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 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, 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):

	Nine Months Ended September 30,	
	2025	2024
Net cash used in operating activities	$ (119,912)	$ (75,597)
Net cash provided by investing activities	172	96,929
Net cash provided by financing activities	196,008	106
Net increase in cash, cash equivalents and restricted cash equivalents	$ 76,268	$ 21,438

Operating Activities

Net cash used in operating activities was $119.9 million for the nine months ended September 30, 2025, reflecting a net loss of $2,418.8 million, offset by changes in operating assets and liabilities of $7.0 million and non-cash charges of $2,291.9 million. The non-cash charges primarily consisted of the change in fair value of warrant liabilities of $2,090.4 million and the issuance of pre-funded warrants in connection with the acquisition of in-process research and development of $177.4 million.

Net cash used in operating activities was $75.6 million for the nine months ended September 30, 2024, reflecting a net loss of $86.2 million and net cash used of $2.8 million for operating assets and liabilities, which were offset by non-cash charges of $13.4 million. The non-cash charges primarily consisted of stock-based compensation expense of $8.1 million, non-cash lease expense of $3.8 million and depreciation expense of $2.7 million, offset by $1.1 million of non-cash interest earned on marketable securities.

The $44.3 million increase in net cash used in operating activities for the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024 was primarily due to the $45.0 million up-front license payment due under the Telitacicept License Agreement.

Investing Activities

Net cash provided by investing activities was $0.2 million for the nine months ended September 30, 2025, which consisted of proceeds from the sales of property and equipment of $0.8 million, partially offset by $0.6 million in purchases of property and equipment. Net cash provided by investing activities was $96.9 million for the nine months ended September 30, 2024, which consisted of proceeds of $107.0 million from the maturity of marketable securities, partially offset by purchases of $9.9 million of marketable securities and $0.2 million of property and equipment.

Financing Activities

Net cash provided by financing activities was $196.0 million for the nine months ended September 30, 2025, which primarily consisted of $175.0 million in proceeds from the issuance of pre-funded warrants, $20.3 million in proceeds from the issuance of common stock under the Stifel ATM Facility, and $2.1 million in proceeds from the exercise of stock options and issuance of shares under the ESPP, partially offset by $1.2 million of issuance costs related to the December 2024 private placement and June 2025 private placement. Net cash provided by financing activities was $0.1 million for the nine months ended September 30, 2024, which consisted of proceeds from the issuance of common stock under the Stifel ATM Facility of $0.3 million and proceeds from exercise of stock options and issuance of shares under the ESPP of $0.1 million, offset by $0.3 million of taxes paid related to net share settlement of equity awards.

Contractual Obligations and Other Commitments

As noted in Note 9 to the unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q, we amended our lease for corporate office and laboratory real estate space to terminate on August 4, 2025. On June 30, 2025, we abandoned such space and have no remaining contractual payments related to the leased space. As further noted in Note 9 to the unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q, on August 12, 2025, we entered into a non-cancellable lease relating to corporate office space, with a term expiring in August 2031.

Other commitments include license and collaboration agreements we have entered into with certain parties including the Telitacicept License Agreement discussed in Note 10 to the unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. 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. Payments under these arrangements will generally be expensed as incurred.

Other than the changes discussed above, during the nine months ended September 30, 2025, there were no additional significant changes in contractual obligations and commitments from that described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations—Contractual Obligations and Other Commitments" in our 2024 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.

Recent Accounting Pronouncements

There are no new significant recent accounting pronouncements which may materially impact our financial statements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company as defined by Item 10 of Regulation S-K and are not required to provide the information otherwise required under this item.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (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 SEC'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, or persons performing similar functions, 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 September 30, 2025, our Chief Executive Officer and our Chief Financial Officer concluded that our disclosure controls and procedures as of such date were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended September 30, 2025 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not currently a party to any material legal proceedings. From time to time, we may become involved in other litigation or legal proceedings relating to claims arising from the ordinary course of business.

Item 1A. Risk Factors.

The following risk factors and other information included in this Quarterly Report on Form 10-Q ("Quarterly Report"), including our condensed consolidated financial statements and related notes thereto, as well as our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 ("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 Quarterly 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 nine months ended September 30, 2025 and 2024 our net loss was $2,418.8 million and $86.2 million, respectively. As of September 30, 2025, we had an accumulated deficit of $2,875.8 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;
- initiate clinical development of telitacicept in additional indications, including Sjögren's disease;
- 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 initiate clinical development of telitacicept in additional indications, including Sjögren's disease, 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 September 30, 2025, our cash, cash equivalents and marketable securities were $170.5 million. We expect that our existing cash, cash equivalents and marketable securities as of September 30, 2025, together with the net proceeds from at-the-market sales during October 2025 and the net proceeds and expected net proceeds from the November 2025 Offering, will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2027. 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, such as our issuance and sale of 11,500,000 shares of common stock in the November 2025 Offering (assuming the closing of the purchase of the additional shares by the underwriters occurs) and sales made pursuant to 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 of warrants to purchase shares of our common stock. 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 September 30, 2025, the 2024 Warrants and RemeGen Warrant remained outstanding and unexercised, and 32,854,170 of the 2025 PIPE Warrants remain outstanding and unexercised. In addition, as of September 30, 2025, we have outstanding options to purchase 5,280,272 shares of common stock and 883,136 restricted stock units, and we have 1,893,219 shares of common stock available for future issuance under our 2023 Inducement Plan, 1,739,508 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 trial for the treatment of gMG. 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, 2024, we had gross federal net operating loss carryforwards of $243.9 million including $242.0 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 $234.7 million which will expire at various dates through 2044. 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 Discovery, 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 a global Phase 3 clinical trial for the treatment of gMG, 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 trial of telitacicept in gMG;
- 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.

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 May 2025, we announced that, based on the available clinical data from our then-key clinical programs and a challenging fundraising environment, we were initiating a process to explore strategic alternatives and winding down our then-ongoing clinical and manufacturing operations and clinical trials. 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 AbbVie, Inc. (Humira/rheumatoid arthritis), Amgen, Inc. (Uplinza/Myasthenia Gravis, Enbrel/rheumatoid arthritis), Argenx (VYVGART/Myasthenia Gravis), Biogen Inc, (Tysabri/multiple sclerosis), GlaxoSmithKline plc (Benlysta/lupus), F. Hoffman-La Roche AG (Rituxan/often used off label) and Johnson & Johnson (Imaavy/Myasthenia Gravis, Remicade/rheumatoid arthritis and Stelara/psoriasis).

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. For example, we are aware of several neonatal Fc receptor (FcRn) inhibitors that are in clinical development and one FcRn inhibitor, Rystiggo (rozanolixizumab-noli), which was approved in June 2023, and another, Imaavy (nipocalimab-aahu), which was approved in April 2025 for the treatment of adults and pediatric patients age 12 and above with gMG who are AChR-AB+ or MuSK-AB+. We are also aware that AstraZeneca PLC is selling Soliris and Ultomiris for the treatment of adult patients with gMG who are AChR-AB+ and that UCB is selling Rystiggo for the treatment of adult patients with gMG who are AChR-AB+ or MuSK-AB+ and Zilbrysq for the treatment of adult patients with gMG who are AChR-AB+. 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).

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, 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.

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 trial of telitacicept in gMG may not be replicated in our ongoing global Phase 3 clinical trial of telitacicept in gMG 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.

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 preclinical development and then conduct 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 for some aspects of our research and preclinical testing, and 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 to conduct some aspects of our research and preclinical testing, and 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 research and development and 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 future preclinical studies and clinical trials will also result in less direct control over the management of data developed through preclinical studies and 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 preclinical studies and 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 preclinical studies and future 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 have contracted and 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 reliance on third parties increases the risk that we will not have sufficient quantities and quality of such materials, product candidates or any products that we may develop and commercialize, 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, as well as our research programs and preclinical studies, and expect to continue to do so for future clinical testing and for commercial supply of our product candidates and 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 preclinical research and 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 priorities under the new administration 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. In addition, the Trump administration has discussed several changes to the reach and oversight of the FDA, which could affect its relationship with the pharmaceutical industry, transparency in decision making and ultimately the cost and availability of prescription drugs. 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.

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 August 16, 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the “donut hole” under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and creating a new manufacturer discount program. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. We cannot predict the ultimate content, timing or effect of any such challenges or changes to the ACA or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare reform will affect our business.

Federal and state governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs. For example, on July 4, 2025, the annual reconciliation bill, the "One Big Beautiful Bill Act" (“OBBBA”) was signed into law which, 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. OBBBA also narrows access to ACA marketplace exchange enrollment and declines to extend the ACA enhanced advanced premium tax credits, set to expire at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. Additionally, the IRA also, among other things, (1) directs the U.S. Department of Health and Human Services (“HHS”), to negotiate the price of certain high expenditure single-source biologics that have been on the market for at least 11 years covered under Medicare (the “Medicare Drug Price Negotiation Program”), and (2) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions began to take effect progressively in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon price of the first ten products that were subject to price negotiations, although the Medicare Drug Price Negotiation Program is currently subject to legal challenges. On January 17, 2025, HHS selected fifteen additional products covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program. Further, on December 7, 2023, an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act was announced. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future, or what effect such legislation, regulations or policies would have on our business. The current Trump administration is pursuing policies to reduce regulations and expenditures across government 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, on September 30, 2025, the current administration announced the first agreement with a major pharmaceutical company that requires the drug manufacturer to offer, through a direct to consumer platform, 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) directives to reduce agency workforce and cut programs; (2) rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation to consider new payment and healthcare models to limit drug spending; (3) eliminating the Biden administration's executive order that directed HHS to establish an AI task force and develop a strategic plan; (4) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (5) imposing tariffs of imported pharmaceutical products; (6) and directing certain federal agencies to enforce existing law regarding hospital and plan price transparency and by standardizing prices across hospitals and health plans and (7) as part of the Make America Healthy Again ("MAHA") Commission's recent Strategy Report, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. 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. Any cost containment measures could significantly decrease the available coverage and the price we might establish for our potential products, which would have an adverse effect on our net revenues and operating results.

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 laws and regulations, which among other things, impose certain requirements relating to the privacy, security and transmission of personal data. 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. 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.

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 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. Regulators 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. 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.

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, 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.

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.

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. We have in the past and may in the future expend significant resources or modify our business 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, penalties and fines, regulatory actions or investigations, 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.

Our shares of common stock began trading on the Nasdaq Global Select Market on February 5, 2021. Given the limited trading history of our common stock, 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.

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 $3.02 per share for the period of January 1, 2025 through November 11, 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 preclinical studies and 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 political instability, including terrorist attacks, civil unrest and actual or threatened armed conflict, such as the Russia-Ukraine and Israel-Hamas wars;
- 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, and this registration statement is expected to become effective in December 2025.

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. In particular, our two largest stockholders, RA Capital Management, L.P. and Reprogrammed Interchange LLC, together with their affiliates, collectively hold approximately 13.7% 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, the Russia-Ukraine and Israel-Hamas 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 $66^{2}/_{3}\%$ 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 5. Other Information

Director and Officer Trading Arrangements

During the quarter ended September 30, 2025, no director or officer, as defined in Rule 16a-1(f), 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 6. 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 to the Amended and Restated Certificate of Incorporation of the Registrant	8-K	001-39979	3.1	May 23, 2025	
3.3	Certificate of Amendment to the 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-3	333-290490	4.5	September 24, 2025	
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	
10.1+	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	
10.2+	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	
10.3*	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	
10.4^	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	
10.5+	Vor Biopharma Inc. Amended and Restated 2021 Equity Incentive Plan	8-K	001-39979	10.1	August 27, 2025	
10.6+	Amendment to Employment Agreement, dated November 2, 2025, by and between the Registrant and Qing Zuraw					X
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) under the Securities Exchange Act of 1934, as amended, 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)/15d-14(a) under the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002					X
32.1†	Certification of Principal Executive and Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002					X
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	The cover page from the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2025, formatted in Inline XBRL.	

+ 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.

† The certifications furnished in Exhibit 32.1 hereto are deemed to be furnished with this Quarterly Report on Form 10-Q and will not be deemed to be "filed" for purposes of Section 18 of the Exchange Act. Such certifications will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that the Registrant specifically incorporates it by reference.

SIGNATURES

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

VOR BIOPHARMA INC.

Date: November 13, 2025

By: /s/ Jean-Paul Kress
Jean-Paul Kress
President and Chief Executive Officer (Principal Executive Officer)

Date: November 13, 2025

By: /s/ Sandy Mahatme
Sandy Mahatme
Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)

Exhibit 10.6

AMENDMENT TO EMPLOYMENT AGREEMENT

This AMENDMENT TO EMPLOYMENT AGREEMENT (this "***Amendment***"), is made and entered into as of November 2, 2025 (the "***Amendment Effective Date***"), by and between Vor Biopharma Inc. (the "***Company***") and Qing Zuraw ("***Executive***"), and amends the Employment Agreement by and between the Company and Executive, dated July 17, 2025 (the "***Existing Agreement***").

In consideration of the promises and the mutual covenants and agreements herein contained, and intending to be legally bound hereby, the Company and Executive agree that the Existing Agreement shall be amended as set forth herein.

1. For five months following the Amendment Effective Date, the following modifications shall be made to Section 6 ("Termination of Employment") of the Existing Agreement:

A. Section 6.1(b)(iii) shall be inserted as follows:

(iii) The Company will pay Executive (1) if the Executive's employment terminates before the date on which 2025 annual bonuses are paid, a pro-rated Target Bonus for 2025 payable in a lump sum; and (2) if applicable, a pro-rated Target Bonus for the year in which Executive's employment terminates, calculated based on the percentage of the year that the Executive worked and payable in lump sum.

B. Section 6.2 shall be replaced in its entirety with the following:

6.2. ***Resignation by Executive for Any Reason (not in connection with a Change in Control).***

(a) Provided that Executive has not previously been notified of the Company's intention to terminate Executive's employment, Executive may resign from employment with the Company for any reason after December 31, 2025.

(b) In the event Executive resigns from Executive's employment, then Executive shall be entitled to the Accrued Obligations and, provided such resignation constitutes a Separation from Service and Executive complies with the obligations in Section 6.1(c) of this Agreement (including the requirement to provide an effective Release), Executive shall also be eligible to receive the same Severance Benefits as described in Section 6.1 and on the same conditions as if Executive had been terminated by the Company without Cause.

C. Section 6.5 shall be null and void.

2. For the avoidance of doubt, in the event that Executive remains employed by the Company through December 31, 2025, her 2025 Annual Discretionary Bonus shall be paid at Target Bonus level (as defined in the Existing Agreement) on a pro-rated basis based on the percentage of 2025 that Executive worked.

3. The modifications contained in Sections 1 and 2 of this Amendment are all expressly conditioned upon Executive's good-faith cooperation and best efforts in performing transition duties as assigned by the Company. Specifically, the Executive agrees to: (i) expend best efforts on behalf of the Company; (ii) refrain from any actions or activities that are reasonably foreseeable to cause the Company harm or hinder the seamless transition of any duties; and (iii) devote full business attention to the performance of any assigned duties, including any reasonable deliverables required by the Company.

4. The reference in Section 6.1(c) of the Existing Agreement to a "post-employment non-competition agreement" is hereby amended to "12-month post-employment non-competition agreement".

5. The provisions of Section 7 (General Provisions) of the Existing Agreement shall apply to this Amendment, *mutatis mutandis*.

6. Except as expressly modified pursuant to this Amendment, the Existing Agreement shall remain in full force and effect in accordance with its terms.

IN WITNESS WHEREOF, the undersigned have executed this Amendment to the Existing Agreement as of the date first written above.

VOR BIOPHARMA, INC. Qing Zuraw

By: /s/ Jean-Paul Kress By: /s/ Qing Zuraw
Name: Jean-Paul Kress
Title: CEO

Exhibit 31.1

CERTIFICATIONS

I, Jean-Paul Kress, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Vor Biopharma Inc.;

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

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

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

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

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

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

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

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

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

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

Date: November 13, 2025

By: /s/ Jean-Paul Kress
Jean-Paul Kress
President and Chief Executive Officer
(*Principal Executive Officer*)

Exhibit 31.2

CERTIFICATIONS

I, Sandy Mahatme, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Vor Biopharma Inc.;

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

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

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

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

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

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

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

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

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

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

Date: November 13, 2025

By: /s/ Sandy Mahatme
Sandy Mahatme
Chief Financial Officer
(*Principal Financial Officer and Principal Accounting Officer)*

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of Vor Biopharma Inc. (the "Company") for the period ended September 30, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

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

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

Date: November 13, 2025

By: /s/ Jean-Paul Kress
Jean-Paul Kress
President and Chief Executive Officer
(*Principal Executive Officer*)

Date: November 13, 2025

By: /s/ Sandy Mahatme
Sandy Mahatme
Chief Financial Officer
(*Principal Financial Officer and Principal Accounting Officer*)

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Vor Biopharma Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of this Form 10-Q), irrespective of any general incorporation language contained in such filing.