

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549**

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended June 30, 2023

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
(State or other jurisdiction of
incorporation or organization)
100 Cambridgepark Drive, Suite 101
Cambridge, Massachusetts
(Address of principal executive offices)

81-1591163
(I.R.S. Employer
Identification No.)

02140
(Zip Code)

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

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

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

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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input checked="" type="checkbox"/>

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 August 4, 2023 was 67,538,680.

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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,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions intended to identify statements about the future. These statements speak only as of the date of this 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 preclinical studies and clinical trials of our 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 and success of our in-house or third-party clinical manufacturing capabilities and efforts;
- the timing of any submission of filings for regulatory approval of, and our ability to obtain and maintain regulatory approvals for, our product candidates for any indication;
- our ability to identify patients with the diseases treated by our product candidates, and to enroll patients in trials;
- our expectations regarding the market acceptance and opportunity for and clinical utility of our 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 candidates;
- our estimates of our expenses, ongoing losses, future revenue, 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 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 competitors 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 “Summary Risk Factors” and “Risk Factors” sections in our Annual Report on Form 10-K for the year ended December 31, 2022 for a discussion of material factors that could cause actual results or events to differ materially from the forward-looking statements that we make.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements (Unaudited).

VOR BIOPHARMA INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(UNAUDITED)

(in thousands, except share amounts)	June 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	\$ 32,212	\$ 57,706
Marketable securities	154,690	172,539
Prepaid expenses	3,952	4,368
Other current assets	539	2,337
Total current assets	<u>191,393</u>	<u>236,950</u>
Restricted cash equivalents	2,413	2,413
Property and equipment, net	11,458	12,634
Operating lease right-of-use assets	42,454	44,444
Other assets	3,268	2,925
Total assets	<u>\$ 250,986</u>	<u>\$ 299,366</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 1,648	\$ 1,772
Accrued liabilities	7,256	7,889
Operating lease liabilities	3,691	3,272
Other current liabilities	122	186
Total current liabilities	<u>12,717</u>	<u>13,119</u>
Long-term liabilities:		
Operating lease liabilities—non-current	33,819	35,640
Total liabilities	<u>46,536</u>	<u>48,759</u>
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 10,000,000 shares authorized as of June 30, 2023 and December 31, 2022; 0 shares issued and outstanding as of June 30, 2023 and December 31, 2022	—	—
Common stock, \$0.0001 par value; 400,000,000 shares authorized as of June 30, 2023 and December 31, 2022; 67,464,265 and 66,079,597 shares issued and 67,433,560 and 65,996,138 outstanding as of June 30, 2023 and December 31, 2022, respectively	7	7
Additional paid-in capital	485,508	473,587
Accumulated other comprehensive loss	(436)	(770)
Accumulated deficit	(280,629)	(222,217)
Total stockholders' equity	<u>204,450</u>	<u>250,607</u>
Total liabilities and stockholders' equity	<u>\$ 250,986</u>	<u>\$ 299,366</u>

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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	\$ 23,897	\$ 15,333	\$ 45,812	\$ 30,613
General and administrative	8,277	6,459	16,784	13,979
Total operating expenses	\$ 32,174	\$ 21,792	\$ 62,596	\$ 44,592
Loss from operations	\$ (32,174)	\$ (21,792)	\$ (62,596)	\$ (44,592)
Other income:				
Interest income	2,195	133	4,184	196
Total other income	2,195	133	4,184	196
Net loss	\$ (29,979)	\$ (21,659)	\$ (58,412)	\$ (44,396)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.45)	\$ (0.58)	\$ (0.88)	\$ (1.19)
Weighted-average common shares outstanding, basic and diluted	67,033,150	37,437,063	66,651,547	37,365,647
Other comprehensive income (loss):				
Unrealized gain (loss) on available for sale investments	(262)	(254)	334	(1,326)
Total other comprehensive income (loss)	(262)	(254)	334	(1,326)
Comprehensive loss	\$ (30,241)	\$ (21,913)	\$ (58,078)	\$ (45,722)

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

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

(in thousands, except share amounts)	Common Stock			Additional Paid-In Capital	Accumulated other comprehensive loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31, 2022	65,996,138	\$	7	\$ 473,587	\$ (770)	\$ (222,217)	\$ 250,607
Issuance of common stock upon vesting of RSUs and exercise of stock options, net of shares withheld for taxes	205,485	—	—	(340)	—	—	(340)
Issuance of common stock from open market sales agreement	733,274	—	—	3,717	—	—	3,717
Stock-based compensation expense	—	—	—	4,068	—	—	4,068
Other comprehensive income	—	—	—	—	596	—	596
Net loss	—	—	—	—	—	(28,433)	(28,433)
Balance at March 31, 2023	66,934,897	\$	7	\$ 481,032	\$ (174)	\$ (250,650)	\$ 230,215
Issuance of common stock upon vesting of RSUs, vesting and exercise of stock options, and issuance of common stock from ESSP, net of shares withheld for taxes	381,775	—	—	(317)	—	—	(317)
Issuance of common stock from open market sales agreement	116,888	—	—	555	—	—	555
Stock-based compensation expense	—	—	—	4,238	—	—	4,238
Other comprehensive loss	—	—	—	—	(262)	—	(262)
Net loss	—	—	—	—	—	(29,979)	(29,979)
Balance at June 30, 2023	67,433,560	\$	7	\$ 485,508	\$ (436)	\$ (280,629)	\$ 204,450

(in thousands, except share amounts)	Common Stock			Additional Paid-In Capital	Accumulated other comprehensive loss	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount					
Balance at December 31, 2021	37,174,741	\$	4	\$ 346,382	\$ —	\$ (130,123)	\$ 216,263
Issuance of common stock upon vesting and exercise of stock options	161,573	—	—	247	—	—	247
Stock-based compensation expense	—	—	—	1,746	—	—	1,746
Other comprehensive loss	—	—	—	—	(1,072)	—	(1,072)
Net loss	—	—	—	—	—	(22,737)	(22,737)
Balance at March 31, 2022	37,336,314	\$	4	\$ 348,375	\$ (1,072)	\$ (152,860)	\$ 194,447
Issuance of common stock upon vesting and exercise of stock options	74,358	—	—	146	—	—	146
Issuance of common stock from open market sales agreement	95,564	—	—	553	—	—	553
Stock-based compensation expense	—	—	—	1,741	—	—	1,741
Other comprehensive loss	—	—	—	—	(254)	—	(254)
Net loss	—	—	—	—	—	(21,659)	(21,659)
Balance at June 30, 2022	37,506,236	\$	4	\$ 350,815	\$ (1,326)	\$ (174,519)	\$ 174,974

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)	Six Months Ended June 30,	
	2023	2022
Cash flows from operating activities		
Net loss	\$ (58,412)	\$ (44,396)
Adjustments to reconcile net loss to net cash used in operations:		
Depreciation expense	1,707	1,001
Amortization of operating lease right-of-use assets	2,317	1,311
Stock-based compensation	8,306	3,487
Interest (amortization) accretion on marketable securities	(2,447)	120
Changes in operating assets and liabilities:		
Operating lease liabilities, net	(1,729)	(7,037)
Prepaid expenses and other current assets	2,214	(728)
Accounts payable and accrued liabilities	19	(1,473)
Other assets	(343)	(181)
Net cash used in operating activities	(48,368)	(47,896)
Cash flow from investing activities		
Purchases of marketable securities	(58,370)	(5,044)
Proceeds from maturities of marketable securities	79,000	3,130
Purchases of property and equipment	(493)	(7,843)
Net cash provided by (used in) investing activities	20,137	(9,757)
Cash flow from financing activities		
Payment of issuance costs related to underwritten public offering and concurrent private placement	(717)	—
Proceeds from the issuance of common stock from at-the-market sales agreement, net of issuance costs	4,201	504
Shares repurchased for tax withholdings upon vesting of restricted stock unit awards	(945)	—
Proceeds from stock option exercises and the issuance of shares under ESPP	198	300
Net cash provided by financing activities	2,737	804
Net decrease in cash, cash equivalents and restricted cash equivalents	(25,494)	(56,849)
Cash, cash equivalents and restricted cash equivalents, beginning of period	\$ 60,119	\$ 122,214
Cash, cash equivalents and restricted cash equivalents, end of period	\$ 34,625	\$ 65,365
Supplemental disclosure of non-cash activities		
Right-of-use assets obtained in exchange for lease obligations	\$ 107	\$ 23,376
Purchases of property and equipment in accounts payable and accrued liabilities	\$ —	\$ 1,331
Financing costs associated with the sale of common stock included in accounts payable and accrued expenses	\$ 24	\$ —

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

(in thousands)	For the Six Months Ended June 30,	
	2023	2022
Cash and cash equivalents	\$ 32,212	\$ 62,952
Restricted cash equivalents	2,413	2,413
Total cash, cash equivalents and restricted cash equivalents as shown on the statements of cash flows	\$ 34,625	\$ 65,365

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 cell and genome engineering company that combines a novel patient engineering approach with targeted therapies to provide a single company solution for patients suffering from hematological malignancies. The Company’s proprietary platform leverages its expertise in hematopoietic stem cell (“HSC”) biology, genome engineering and targeted therapy development to genetically modify HSCs to remove surface targets expressed by cancer cells. The Company is headquartered in Cambridge, 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 preclinical studies and clinical trials, dependence on key personnel, protection of proprietary technology, reliance on third party organizations, risks of obtaining regulatory approval for any product candidate that it may develop, development by competitors of technological innovations, compliance with government regulations, geopolitical tensions, adverse macroeconomic conditions and the need to obtain additional financing.

The Company anticipates that it will continue to incur significant operating losses for the next several years as it continues to develop its product candidates. The Company believes that its existing cash, cash equivalents and marketable securities at June 30, 2023 will be sufficient to allow the Company to fund its current operations through at least a period of one year after the date the financial statements are issued.

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”). Certain comparative amounts have been reclassified to conform to the current period presentation. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification and Accounting Standards Update (“ASU”) of the Financial Accounting Standards Board (“FASB”).

Use of Estimates

The preparation of the condensed consolidated financial statements in conformity with GAAP requires the Company’s management to make estimates and assumptions that affect the reported amounts of assets and liabilities at the date of the 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 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, 2022 has been derived from the Company’s audited consolidated financial statements for the year ended December 31, 2022. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to rules and regulations. 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 notes in the Company's Annual Report on Form 10-K for the year ended December 31, 2022 (the "2022 Annual Report").

During the six months ended June 30, 2023, there have been no changes to the Company's significant accounting policies as described in the 2022 Annual Report.

3. Marketable Securities

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

(in thousands)	June 30, 2023			
	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Maturing in one year or less				
U.S. Treasuries	\$ 155,126	\$ 10	\$ (446)	\$ 154,690
Total	<u>\$ 155,126</u>	<u>\$ 10</u>	<u>\$ (446)</u>	<u>\$ 154,690</u>
Maturing after one year through five years				
Corporate bonds	\$ 5,001	\$ —	\$ (56)	\$ 4,945
U.S. Treasuries	116,432	—	(617)	115,815
U.S. Treasuries	51,876	—	(97)	51,779
Total	<u>\$ 173,309</u>	<u>\$ —</u>	<u>\$ (770)</u>	<u>\$ 172,539</u>

The unrealized losses of the Company's marketable securities above were a result of market interest rate increases. The contractual terms of those investments do not permit the issuer to settle the securities at a price less than par value. The Company's intent is to hold the investments until their maturity and any change in fair value that is not credit related is recognized as other comprehensive income (loss), net of applicable taxes. A credit-related impairment is recognized as an allowance to the balance sheet with a corresponding adjustment to earnings. The Company did not recognize any credit losses related to marketable securities for the six months ended June 30, 2023.

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)	June 30, 2023			
	Level 1	Level 2	Level 3	Total
Cash equivalents				
Money market funds	\$ 31,543	\$ —	\$ —	\$ 31,543
Marketable securities				
U.S. Treasuries	—	154,690	—	154,690
Total marketable securities	—	154,690	—	154,690
Restricted cash equivalents				
Money market funds	2,413	—	—	2,413
Total	<u>\$ 33,956</u>	<u>\$ 154,690</u>	<u>\$ —</u>	<u>\$ 188,646</u>

(in thousands)	December 31, 2022			
	Level 1	Level 2	Level 3	Total
Cash equivalents				
Money market funds	\$ 46,981	\$ —	\$ —	\$ 46,981
Marketable securities				
Corporate bonds	—	4,945	—	4,945
U.S. Treasuries	—	167,594	—	167,594
Total marketable securities	—	172,539	—	172,539
Restricted cash equivalents				
Money market funds	2,413	—	—	2,413
Total	\$ 49,394	\$ 172,539	\$ —	\$ 221,933

The fair value of the Company's cash equivalents and restricted cash equivalents is based on quoted market prices in active markets with no valuation adjustment. The fair value of investments was determined based on observable market inputs. There were no transfers between levels during the six months ended June 30, 2023.

Cash equivalents, restricted cash, 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)	June 30, 2023	December 31, 2022
Laboratory equipment	\$ 9,937	\$ 9,499
Manufacturing equipment	6,817	5,706
Computer equipment	432	432
Furniture, fixtures and other	568	568
Construction in progress	11	1,039
Total	17,765	17,244
Less: Accumulated depreciation	(6,307)	(4,610)
Property and equipment, net	\$ 11,458	\$ 12,634

Depreciation expense for the three and six months ended June 30, 2023 was \$0.9 million and \$1.7 million, respectively, and for the three and six months ended June 30, 2022 was \$0.6 million and \$1.0 million, respectively.

6. Accrued Liabilities

Accrued liabilities consisted of the following:

(in thousands)	June 30, 2023	December 31, 2022
Employee-related expenses	\$ 3,123	\$ 4,408
Professional fees	1,318	1,701
Clinical expenses	1,241	532
Research and development expenses	473	569
Manufacturing expenses	955	328
Other	146	351
Total accrued liabilities	\$ 7,256	\$ 7,889

7. Stock-Based Compensation

Stock Incentive Plan

As of June 30, 2023, the Company had 796,957 shares of its common stock available for future issuance under its stock incentive plan.

Stock Options

The Company's stock options generally vest over 48 months with 25% vesting after one year followed by ratable monthly vesting over the remaining three years and have a contractual term of 10 years. The weighted-average assumptions used principally in determining the fair value of options granted were as follows:

	Six Months Ended June 30,			
	2023		2022	
Fair value of common stock	\$	3.76	\$	8.30
Expected term (in years)		6.0		6.0
Expected volatility		81.9%		78.4%
Risk-free interest rate		3.7%		1.8%
Dividend yield		—		—

During the six months ended June 30, 2023 and 2022, the Company granted stock options to purchase 2,108,219 shares and 1,948,264 shares of its common stock, respectively, with a weighted-average grant-date fair value of \$3.76 and \$5.65 per share, respectively. As of June 30, 2023, total unrecognized compensation expense related to stock options was \$19.2 million, which is expected to be recognized over a weighted-average period of 2.4 years.

As of June 30, 2023, options for 30,704 shares of Company common stock with a weighted-average exercise price of \$1.36 were exercised and unvested. The underlying proceeds from the unvested exercises of \$0.1 million is recorded in other current liabilities on the condensed consolidated balance sheet.

Restricted Stock Units

During the six months ended June 30, 2023 and 2022, the Company granted 645,360 restricted stock units and 1,040,500 restricted stock units, respectively, with a weighted-average grant date fair value of \$5.55 and \$6.00 per share, respectively. As of June 30, 2023, total unrecognized compensation expense related to restricted stock units was \$6.2 million, which is expected to be recognized over a weighted-average period of 1.8 years.

Employee Stock Purchase Plan

As of June 30, 2023, the Company had 666,535 shares of its common stock available for issuance under its Employee Stock Purchase Plan ("ESPP").

During the six months ended June 30, 2023, the Company issued 44,977 shares with a weighted-average purchase price of \$3.76 under the ESPP, which resulted in an immaterial amount of compensation expense. The Company did not issue any shares under the ESPP during the six months ended June 30, 2022.

Stock-Based Compensation

Stock-based compensation expense was allocated as follows:

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Research and development	\$ 2,463	\$ 909	\$ 4,817	\$ 1,705
General and administrative	1,775	832	3,489	1,782
Total stock-based compensation expense	\$ 4,238	\$ 1,741	\$ 8,306	\$ 3,487

8. Leases

Cambridgepark Lease Amendments

On June 15, 2021, the Company entered into the first lease amendment ("First Lease Amendment") and second lease amendment ("Second Lease Amendment" and, together with the First Lease Amendment, the "Lease Amendments") with PPF Off 100 Cambridge Park Drive, LLC (the "Landlord"). The Lease Amendments amended the Company's lease agreement for its corporate office and laboratory facilities with the Landlord in Cambridge, Massachusetts to add additional leased space in the same building (the "Amended Cambridgepark Lease").

The First Lease Amendment and Second Lease Amendment commenced for accounting purposes on January 28, 2022 and April 29, 2022, respectively. The terms of the Lease Amendments are through September 2030 for approximately \$8.4 million and \$22.3 million in fixed payments for the First Lease Amendment and Second Lease Amendment, respectively. There are no options to extend the Lease Amendments.

Payments due associated with the Lease Amendments include fixed and variable payments. Variable payments relate to the Company's share of the Landlord's operating costs associated with the underlying assets and are recognized when the event on which those payments are assessed. The Amended Cambridgepark Lease does not contain a residual value guarantee. The Lease Amendments term end dates are coterminous with the existing lease agreement. In conjunction with the Lease Amendments, the Company was required to increase its irrevocable standby letter of credit to \$2.4 million for the benefit of the Landlord, which has been secured by money market investments and is presented as restricted cash equivalents.

For further information regarding the Company's Cambridgepark lease, please see Note 9 to the consolidated financial statements included in the 2022 Annual Report.

The elements of lease expense were as follows:

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Operating lease cost	\$ 1,948	\$ 1,592	\$ 3,901	\$ 2,512
Variable lease cost	661	301	1,258	642
Total lease cost	\$ 2,609	\$ 1,893	\$ 5,159	\$ 3,154

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)	June 30, 2023	December 31, 2022
Assets		
Operating lease right-of-use assets	\$ 42,454	\$ 44,444
Liabilities		
Operating lease liabilities, current	\$ 3,691	\$ 3,272
Operating lease liabilities, non-current	33,819	35,640
Total lease liabilities	\$ 37,510	\$ 38,912
Weighted-Average Lease Term and Discount Rate		
Weighted-average remaining lease term (years)	7.2	7.7
Weighted-average discount rate	8.2%	8.2%

The following table represents other lease activity:

(in thousands)	Six Months Ended June 30,	
	2023	2022
Other Information		
Cash paid for amounts included in the measurement of lease liabilities		
Operating cash flows for operating leases	\$ 3,312	\$ 8,522
Right-of-use assets obtained in exchange for lease obligations	\$ 107	\$ 23,376

9. Significant Agreements

Since December 31, 2022, there have been no material changes to the key terms of the Company's license agreements. For further information regarding the Company's existing license agreements, please see Note 10 to the consolidated financial statements included in the 2022 Annual Report.

10. 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 six months ended June 30, 2023 and 2022:

(in thousands, except share and per share amounts)	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Numerator:				
Net loss attributable to common stockholders	\$ (29,979)	\$ (21,659)	\$ (58,412)	\$ (44,396)
Denominator:				
Weighted-average number of common shares outstanding, basic and diluted	67,033,150	37,437,063	66,651,547	37,365,647
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.45)	\$ (0.58)	\$ (0.88)	\$ (1.19)

The Company's potentially dilutive securities were stock options, unvested restricted stock and restricted stock units. Based on the amounts outstanding as of June 30, 2023 and 2022, 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 June 30,	
	2023	2022
Options to purchase common stock	8,368,063	5,893,016
Unvested restricted stock	30,704	138,052
Restricted stock units	1,324,361	1,040,500

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 our Annual Report on Form 10-K for the year ended December 31, 2022 (the “2022 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 2022 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

We are a clinical-stage company with a vision to cure blood cancers through cell and genome engineering. Our mission is to change the standard of care for patients with blood cancer by engineering hematopoietic stem cells (“HSCs”) to enable the use of targeted therapies post-transplant. Leveraging our expertise in HSC biology and genome engineering, we genetically modify HSCs to remove surface targets and then provide these cells as hematopoietic stem cell transplants to patients. Once these cells engraft into bone marrow, the patient’s healthy cells are protected because they no longer express the surface target, leaving only the cancerous cells exposed. We believe this will unlock the potential of targeted therapies to selectively destroy cancerous cells while sparing healthy cells. As a result, our engineered HSCs (“eHSCs”) are designed to limit the on-target toxicities associated with these targeted therapies, thereby enhancing their utility, and broadening their applicability. We intend to pair future eHSC product candidates with targeted therapeutics such as our VCAR33 programs, chimeric antigen receptor (“CAR”)-T therapies designed to target CD33, as well as with potentially best-in-class targeted therapies from collaborators, to bring potentially transformative outcomes to patients and establish new standard of care Treatment Systems for blood cancers.

We are developing our lead eHSC product candidate, trem-cel (formerly VOR33), which we believe has the potential to transform the treatment for acute myeloid leukemia (“AML”) and other blood cancers. Trem-cel is created by genetically modifying healthy donor HSCs in order to remove the CD33 surface target. We intend to develop trem-cel as a hematopoietic stem cell transplant product candidate to replace the standard of care in transplant settings. Our investigational new drug (“IND”) application for trem-cel in patients with AML was cleared by the U.S. Food and Drug Administration (“FDA”) in January 2021, and we have initiated and are actively recruiting for VBP101, our first-in-human Phase 1/2a trial of trem-cel in combination with Mylotarg. We released clinical data for this trial most recently in June 2023, and we expect to release additional engraftment and hematologic protection data updates by year-end 2023. If successful, this trial will provide important validating evidence of the potential of trem-cel and our broader eHSC approach.

The VCAR33 programs are CAR-T therapy candidates designed to target CD33, a clinically-validated target for AML, that we have licensed from the National Institutes of Health (“NIH”). VCAR33 is made up of two programs with different cell sources. The first uses autologous cells from each patient and is being studied in an ongoing Phase 1/2 clinical trial sponsored by the National Marrow Donor Program (“NMDP”) in young adult and pediatric patients with relapsed/refractory AML in a bridge-to-transplant study, which we refer to as VCAR33^{AUTO}.

The second uses allogeneic healthy donor-derived cells that we refer to as VCAR33^{ALLO}. Our IND application for VCAR33^{ALLO} was cleared by the FDA in June 2023, allowing us to proceed with a planned Phase 1/2 clinical trial for patients with relapsed/refractory AML. The Phase 1/2 clinical trial, VBP301, will enroll patients who have relapsed following allogeneic stem cell transplant, which uses lymphoid cells harvested from the original donor as starting material for the drug product.

The NMDP is also currently evaluating VCAR33^{AUTO} in a multi-site Phase 1/2 clinical trial in young adult and pediatric patients with relapsed/refractory AML. The timing of the data release is dependent on the investigators conducting the trial. The NMDP is responsible for all aspects of the VCAR33^{AUTO} trial, including the design of the trial, the manufacture of study product, the enrollment, dosing and follow-up of patients, the recording of trial data and the analysis of results. We did not control the preclinical development of VCAR33^{AUTO}, which was conducted by the NIH, and we do not have rights under the license agreement to certain intellectual property, such as know-how, employed by the NMDP in manufacturing study product or conducting its clinical trial, however, the NMDP has permitted us to cross-reference its IND for this trial in IND applications that we submit with the FDA.

We believe that the combination of trem-cel followed by treatment with VCAR33^{ALLO} in the post-transplant setting, which we refer to as the trem-cel + VCAR33 Treatment System, may transform patient outcomes and offer the potential for cures for patients that have limited treatment options. The trem-cel + VCAR33 Treatment System would utilize the same healthy donor allogeneic cell source for both trem-cel and VCAR33^{ALLO}. Following ongoing discussions with the FDA and alongside improved scientific understanding of the differences in T-cell sources, we plan to collect initial data on trem-cel from the VBP101 clinical trial and initial clinical data from the VCAR33^{ALLO} program prior to IND submission for the Treatment System. The VBP301 protocol allows for patients who have received a trem-cel transplant on the VBP101 study to enroll onto VBP301 and receive VCAR33^{ALLO}. This may provide valuable early insights into the potential of the Treatment System combining trem-cel and VCAR33^{ALLO} to enable a more

potent therapy and durable responses post-transplant. We believe this approach allows for a more methodical development pathway for this novel-novel treatment combination.

Our new in-house manufacturing facility recently completed Current Good Manufacturing Practices (cGMP) qualification activities and is now able to initiate clinical manufacturing of VCAR33^{ALLO} for the VBP301 trial. The first engineering runs required in preparation for tech transfer of trem-cel to our in-house facility have been completed. We are on-track to commence in-house trem-cel manufacturing in 2023 and will continue to leverage a third-party to provide manufacturing redundancy.

We recently secured a worldwide non-exclusive license from Editas Medicine for ex vivo Cas9 gene-edited HSC therapies for the treatment and/or prevention of hematological malignancies. The license provides access to key intellectual property for the continued development and potential commercialization of edited HSCs including trem-cel, with the option to elect additional product candidate targets within the next five years.

Since our inception in December 2015, we have devoted substantially all of our resources to raising capital, organizing and staffing our company, business and scientific planning, conducting discovery and research activities, acquiring or discovering product candidates, establishing and protecting our intellectual property portfolio, developing and progressing our product candidates and preparing for clinical trials, establishing arrangements with third parties for the manufacture of our product candidates and component materials, building out our internal clinical manufacturing facility, and providing general and administrative support for these operations. We do not have any product candidates approved for sale and have not generated any revenue from product sales. Through June 30, 2023, we funded our operations primarily through the sale of equity securities and debt financings and have received aggregate net proceeds from these transactions of \$463.5 million.

We have incurred significant operating losses since inception, including net losses of \$30.0 million and \$58.4 million for the three and six months ended June 30, 2023, and \$92.1 million for the year ended December 31, 2022. As of June 30, 2023, we had an accumulated deficit of \$280.6 million.

As of June 30, 2023, we had cash, cash equivalents and marketable securities of \$186.9 million. We expect that our cash, cash equivalents and marketable securities at June 30, 2023 will enable us to fund our operating expenses and capital expenditure requirements into the first quarter of 2025.

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. There have been no 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 2022 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 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 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 CMOs; and
- payments made 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.

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 stage of development, preclinical or clinical. However, we do not track our internal research and development expenses on a program specific basis because these costs are deployed across multiple projects and, as such, are not separately classified.

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 more of our product candidates move into clinical development and 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 preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to maintain our current research and development programs and to establish new ones;
- establishing an appropriate safety profile with IND-enabling studies;
- the number of sites and patients included in the clinical trials;
- the countries in which the clinical trials are conducted;
- per patient trial costs;
- successful patient enrollment in, and the initiation of, clinical trials, as well as drop out or discontinuation rates;
- the successful completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the number of trials required for regulatory approval;
- the timing, receipt and terms of any regulatory approvals from applicable regulatory authorities;
- our ability to establish new licensing or collaboration arrangements;
- the performance of our current and future collaborators, if any;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- significant and changing government regulation and regulatory guidance;

- the impact of any business interruptions to our operations or to those of the third parties with whom we work;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- launching commercial sales of our product candidates, if approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of the product candidates following approval.

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

General and Administrative Expenses

General and administrative expenses consist primarily of personnel-related costs, including salaries, bonuses, benefits and stock-based compensation expenses for employees involved in our executive, finance, corporate, business development and administrative functions, as well as expenses for outside professional services, including legal, audit, accounting and tax-related services and other consulting fees, facility-related expenses, which include depreciation costs and other allocated expenses for rent and maintenance of facilities, insurance costs, recruiting costs, travel expenses and other general administrative expenses.

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

Other Income

Interest Income

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

Results of Operations

Comparison of Three and Six Months Ended June 30, 2023 and 2022

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

	Three Months Ended June 30,		Change
	2023	2022	
Operating expenses:			
Research and development	\$ 23,897	\$ 15,333	\$ 8,564
General and administrative	8,277	6,459	1,818
Total operating expenses	32,174	21,792	10,382
Loss from operations	(32,174)	(21,792)	(10,382)
Other income:			
Interest income	2,195	133	2,062
Total other income	2,195	133	2,062
Net loss	\$ (29,979)	\$ (21,659)	\$ (8,320)

	Six Months Ended June 30,		Change
	2023	2022	
Operating expenses:			
Research and development	\$ 45,812	\$ 30,613	\$ 15,199
General and administrative	16,784	13,979	2,805
Total operating expenses	62,596	44,592	18,004
Loss from operations	(62,596)	(44,592)	(18,004)
Other income:			
Interest income	4,184	196	3,988
Total other income	4,184	196	3,988
Net loss	\$ (58,412)	\$ (44,396)	\$ (14,016)

Research and Development Expenses

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

	Three Months Ended June 30,		Change
	2023	2022	
External expenses	\$ 10,762	\$ 6,019	\$ 4,743
Internal expenses:			
Personnel expenses (including stock-based compensation)	9,375	6,928	2,447
Facilities and other expenses	3,760	2,386	1,374
Total research and development expenses	<u>\$ 23,897</u>	<u>\$ 15,333</u>	<u>\$ 8,564</u>

	Six Months Ended June 30,		Change
	2023	2022	
External expenses	\$ 20,018	\$ 12,523	\$ 7,495
Internal expenses:			
Personnel expenses (including stock-based compensation)	18,423	13,887	4,536
Facilities and other expenses	7,371	4,203	3,168
Total research and development expenses	<u>\$ 45,812</u>	<u>\$ 30,613</u>	<u>\$ 15,199</u>

Research and development expenses were \$23.9 million for the three months ended June 30, 2023, compared to \$15.3 million for the three months ended June 30, 2022. The increase of \$8.6 million was primarily due to an increase in clinical, manufacturing and consulting expenses of \$4.7 million as a result of the ongoing trem-cel clinical trial and the development of our VCAR33 programs, an increase in personnel expenses of \$2.5 million, including an increase in stock-based compensation expense of \$1.6 million, and an increase in facility costs from our laboratory and cGMP manufacturing facility expansion of \$1.4 million.

Research and development expenses were \$45.8 million for the six months ended June 30, 2023, compared to \$30.6 million for the six months ended June 30, 2022. The increase of \$15.2 million was primarily due to an increase in clinical, manufacturing and consulting expenses of \$7.5 million as a result of the ongoing trem-cel clinical trial and the development of our VCAR33 programs, an increase in personnel expenses of \$4.5 million, including an increase in stock-based compensation expense of \$3.1 million, attributable to an increase in employee headcount to support the growth of our research and development efforts, and an increase in facility costs from our laboratory and cGMP manufacturing facility expansion of \$3.2 million.

General and Administrative Expenses

General and administrative expenses were \$8.3 million for the three months ended June 30, 2023, compared to \$6.5 million for the three months ended June 30, 2022. The increase of \$1.8 million was primarily due to an increase in personnel costs of \$1.4 million, including an increase in stock-based compensation expense of \$1.0 million, and an increase in facilities and other expenses of \$0.4 million.

General and administrative expenses were \$16.8 million for the six months ended June 30, 2023, compared to \$14.0 million for the six months ended June 30, 2022. The increase of \$2.8 million was primarily due to increased personnel expenses of \$2.3 million, including an increase in stock-based compensation expense of \$1.7 million, and an increase in professional fees of \$0.5 million.

Other Income

Other income increased by \$2.1 million during the three months ended June 30, 2023, compared to the three months ended June 30, 2022. Other income increased by \$4.0 million for the six months ended June 30, 2023, compared to the six months ended June 30, 2022. The increase in other income in both periods was due to increases in interest earned from our cash, cash equivalents and marketable securities held at financial institutions.

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 debt financings and have received aggregate net proceeds from these transactions of \$463.5 million as of June 30, 2023.

In order to fund our future operations, including our planned clinical trials, on March 14, 2022, 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. The Shelf Registration Statement was declared effective by the SEC on March 18, 2022. We believe that our Shelf Registration Statement provides us with the flexibility to raise additional capital to finance our operations as needed. In December 2022, we issued 15,302,267 shares of common stock in an underwritten public offering under our Shelf Registration Statement at a price per share of \$4.30 for proceeds of \$61.3 million, after deducting underwriting discounts, commissions, and offering expenses payable by us. In a separate concurrent private placement, we sold 11,627,907 shares of common stock at a price of \$4.30 per share to RA Capital Healthcare Fund, L.P. for proceeds of \$49.5 million, after deducting related placement fees payable by us. We may offer additional securities under our Shelf Registration Statement from time to time in response to market conditions or other circumstances if we believe such a plan of financing is in the best interests of our stockholders.

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 850,162 shares of our common stock under the Stifel ATM Facility during the six months ended June 30, 2023 at a weighted-average price per share of \$5.11 for aggregate net proceeds of \$4.3 million, after deducting commissions. As of June 30, 2023, \$120.6 million remained available to be sold under the Stifel ATM Facility.

In February 2021, we closed our initial public offering of 11,302,219 shares of our common stock at a public offering price of \$18.00 per share for aggregate net proceeds of \$186.3 million, after deducting underwriting discounts and commissions and other offering expenses payable by us.

Cash Requirements

As of June 30, 2023, there were no material changes in our short-term and long-term cash requirements from those disclosed in the "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in our 2022 Annual Report.

As of June 30, 2023, we had cash, cash equivalents and marketable securities of \$186.9 million. We will need to raise additional capital in the future to fund our 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.

We expect that our cash, cash equivalents and marketable securities at June 30, 2023 will enable us to fund our operating expenses and capital expenditure requirements into the first quarter of 2025. 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 preclinical and clinical development of our product candidates, including in particular the expenses associated with our clinical trials;
- incur third party manufacturing costs to support our preclinical studies and clinical trials of our product candidates and, if approved, their commercialization;
- seek to identify and develop additional product candidates;
- make investments in our platform, including the costs of developing internal manufacturing capabilities;
- seek regulatory and marketing approvals for our 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, technologies;
- maintain, expand, enforce, defend and protect our intellectual property;
- hire additional clinical, quality control, manufacturing and other scientific personnel;

- add operational, financial and management information systems and personnel;
- expand our office, laboratory and manufacturing facility; and
- experience any delays or encounter any issues with any of the above.

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

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through the public or private sale of our equity, government or private party grants, debt financings or other capital sources, including potential collaborations with other companies or other strategic transactions. To the extent that we raise additional capital through the sale of our equity or convertible debt securities, including through the use of the Stifel ATM Facility, the ownership interest of our stockholders will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we 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, changes in financial condition, revenues or expenses, results of operations, liquidity, cash requirements or capital resources.

Cash Flows

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

	Six Months Ended June 30,	
	2023	2022
Net cash used in operating activities	\$ (48,368)	\$ (47,896)
Net cash provided by (used in) investing activities	20,137	(9,757)
Net cash provided by financing activities	2,737	804
Net decrease in cash, cash equivalents and restricted cash equivalents	<u>\$ (25,494)</u>	<u>\$ (56,849)</u>

Operating Activities

Net cash used in operating activities was \$48.4 million for the six months ended June 30, 2023, reflecting a net loss of \$58.4 million and net cash use of \$0.2 million for operating assets and liabilities, which were offset by non-cash charges of \$9.9 million. The non-cash charges primarily consisted of stock-based compensation expense of \$8.3 million, the amortization of operating lease right-of-use assets of \$2.3 million and depreciation expense of \$1.7 million, offset by \$2.4 million of non-cash interest earned on marketable securities.

Net cash used in operating activities was \$47.9 million for the six months ended June 30, 2022, reflecting a net loss of \$44.4 million and net cash used of \$9.4 million for operating assets and liabilities, that were partially offset by non-cash charges of \$5.9 million. The non-cash charges primarily consisted of stock-based compensation expense of \$3.5 million, the amortization of operating lease right-of-use assets of \$1.3 million and depreciation expense of \$1.0 million.

The \$0.5 million decrease in net cash used in operating activities for the six months ended June 30, 2023 compared to the six months ended June 30, 2022 was primarily due to differences in the timing of payments for research and development expenses incurred during each respective period.

Investing Activities

Net cash provided by investing activities was \$20.1 million for the six months ended June 30, 2023, which consisted of purchases of \$58.4 million of marketable securities and \$0.5 million of property and equipment offset by proceeds of \$79.0 million from the maturity of marketable securities. Net cash used in investing activities was \$9.8 million for the six months ended June 30, 2022, which consisted of purchases of \$5.1 million of marketable securities and \$7.8 million of purchases of property and equipment, offset by proceeds of \$3.1 million from the maturity of marketable securities.

Financing Activities

Net cash provided by financing activities was \$2.7 million for the six months ended June 30, 2023, which consisted of proceeds from the issuance of common stock under the Stifel ATM Facility of \$4.2 million, and proceeds from stock option exercises and purchases of common stock under the ESPP of \$0.2 million offset by the payment of \$0.7 million of issuance costs related to the underwritten public offering under our Shelf Registration Statement and concurrent private placement that closed in December 2022, and \$0.9 million of taxes paid related to net share settlement of equity awards. Net cash provided by financing activities was \$0.8 million for the six months ended June 30, 2022, which consisted of proceeds from the issuance of common stock under an open market sale agreement of \$0.5 million, and proceeds from stock option exercises of \$0.3 million.

Contractual Obligations and Other Commitments

Contractual obligations relate to future minimum lease payments for existing non-cancellable leases primarily relating to corporate office and laboratory real estate, with terms expiring through February 2030. During the six months ended June 30, 2023, there were no 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 2022 Annual Report.

Other commitments include license and collaboration agreements we have entered into with certain parties. Such arrangements require ongoing payments, including payments upon the achievement of certain development, regulatory and commercial milestones, receipt of sublicense income, as well as royalties on commercial sales. Payments under these arrangements are expensed as incurred.

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.

Interest Rate Risk

Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our exposure to money market funds, corporate debt and U.S. Treasury securities in our cash equivalents and marketable security balances. Interest income is sensitive to changes in the general level of interest rates. However, due to the short-term maturities of our cash equivalents and marketable securities, we believe a hypothetical 100 basis point increase or decrease in interest rates during any of the periods presented would not have had a material impact on our financial statements.

As of June 30, 2023 and December 31, 2022, we had no debt outstanding and therefore were not exposed to related interest rate risk.

Foreign Currency Exchange Risk

All of our employees and our operations are currently located in the United States and our expenses are generally denominated in U.S. dollars. We therefore are not currently exposed to significant market risk related to changes in foreign currency exchange rates.

However, we have contracted with and may continue to contract with non-U.S. vendors who we may pay in local currency. Our operations may be subject to fluctuations in foreign currency exchange rates in the future. To date, foreign currency transaction gains and losses have not been material to our financial statements, and we have not had a formal hedging program with respect to foreign currency. We believe a hypothetical 100 basis point increase or decrease in exchange rates during any of the periods presented would not have a material effect on our financial statements.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We believe that inflation has not had a material effect on our financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and 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 June 30, 2023, our Chief Executive Officer and 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 June 30, 2023 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.

There have been no material changes to the risk factors disclosed in “Part I. Item 1A. Risk Factors” in our 2022 Annual Report.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Use of Proceeds from Registered Securities

On February 9, 2021, we closed our initial public offering of 11,302,219 shares of our common stock, including 1,474,202 shares of our common stock pursuant to the full exercise by the underwriters of an option to purchase additional shares, at a public offering price of \$18.00 per share for aggregate offering proceeds of approximately \$203.4 million (the “IPO”). The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-252175), which was declared effective by the SEC on February 4, 2021, and a registration statement on Form S-1 (File No. 333-252766), which was deemed effective on February 5, 2021.

We received aggregate net proceeds from the IPO of \$186.3 million, after deducting underwriting discounts and commissions and other offering expenses payable by us.

As of June 30, 2023, we have used \$121.6 million of the net proceeds from our IPO primarily to fund the development of trem-cel (formerly VOR33), the development of VCAR33^{ALLO}, VCAR33^{AUTO} and the trem-cel + VCAR33 Treatment System, and the continued expansion of our platform technology, including to advance the research and development of additional eHSC and internal CAR-T programs, as well as for working capital and general corporate purposes.

We have invested the remaining net proceeds from the offering in a variety of capital preservation investments, including short-term investment grade interest bearing instruments and U.S. government securities. There has been no material change in our planned use of the net proceeds from the offering as described in the final prospectus for our IPO filed with the SEC pursuant to Rule 424(b) under the Securities Act.

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference				
		Form	File No.	Exhibit Number	Filing Date	Filed Herewith
3.1	Amended and Restated Certificate of Incorporation of the Registrant	8-K	001-39979	3.1	February 9, 2021	
3.2	Amended and Restated Bylaws of the Registrant	8-K	001-39979	3.2	February 9, 2021	
4.1	Form of Common Stock Certificate of the Registrant	S-1/A	333-252175	4.1	February 1, 2021	
4.2	Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated June 30, 2020	S-1/A	333-252175	4.2	February 1, 2021	
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 Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002					X
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 June 30, 2023, formatted in Inline XBRL.					

† 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: August 10, 2023

By: /s/ Robert Ang
Robert Ang
President and Chief Executive Officer (Principal Executive Officer)

Date: August 10, 2023

By: /s/ Nathan Jorgensen
Nathan Jorgensen
Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)

CERTIFICATIONS

I, Robert Ang, 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(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 10, 2023

By: /s/ Robert Ang
Robert Ang
President and Chief Executive Officer
(Principal Executive Officer)

CERTIFICATIONS

I, Nathan Jorgensen, 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(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 10, 2023

By: /s/ Nathan Jorgensen
Nathan Jorgensen
Chief Financial Officer
(Principal Financial Officer)

**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 June 30, 2023, 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; 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: August 10, 2023

By: /s/ Robert Ang

Robert Ang
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 10, 2023

By: /s/ Nathan Jorgensen

Nathan Jorgensen
Chief Financial Officer
(Principal Financial 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.
