UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

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

For the quarterly period ended March 31, 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: 001-37923

CRISPR THERAPEUTICS AG

(Exact name of registrant as specified in its charter)

Switzerland	**Not Applicable**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
Baarerstrasse 14 6300 Zug, Switzerland	**Not Applicable**
(Address of principal executive offices)	**(Zip Code)**

+41 (0)41 561 32 77
(Registrant's telephone number, including area code)

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 Shares, nominal value CHF 0.03	**CRSP**	**The Nasdaq Global 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, a smaller reporting company, or emerging growth company. See definition 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 registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of May 5, 2023, there were 78,935,098 shares of registrant's common shares outstanding.

Throughout this Quarterly Report on Form 10-Q, the "Company," "CRISPR," "CRISPR Therapeutics," "we," "us," and "our," except where the context requires otherwise, refer to CRISPR Therapeutics AG and its consolidated subsidiaries.

"CRISPR Therapeutics® " standard character mark and design logo, "COBALT™ ,""CRISPRX™ ," "CRISPR TX™ ," "CTX001™ ," "CTX110® ," "CTX112™ ," "CTX130™ ," "CTX131™ ," "CTX310™ ," "CTX320™ ," "VCTX210™" and "VCTX211™ ," are trademarks and registered trademarks of CRISPR Therapeutics AG. All other trademarks and registered trademarks contained in this Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, trademarks, service marks and trade names referred to in this Quarterly Report on Form 10-Q may appear without the ® or ™ symbols and any such omission is not intended to indicate waiver of any such rights.

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 are forward-looking statements. These statements are often identified by the use of words such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "predict," "project," "potential," "will," "would" or the negative or plural of these words or similar expressions or variations, although not all forward-looking statements contain these identifying words. Forward-looking statements in this Quarterly Report on Form 10-Q include, but are not limited to, statements about:

- the safety, efficacy and clinical progress of our various clinical programs, including those for exa-cel (formerly known as CTX001), CTX110, CTX112, CTX130, CTX131, VCTX210 and VCTX211;
- the status of clinical trials, development timelines, regulatory submissions and discussions with regulatory authorities related to product candidates under development by us and our collaborators;
- the initiation, timing, progress and results of our preclinical studies and clinical trials, including our ongoing clinical trials and any planned clinical trials, and our research and development programs, including delays or disruptions in clinical trials, non-clinical experiments and Investigational New Drug, or IND, application-enabling studies;
- the actual or potential benefits of U.S. Food and Drug Administration, or FDA, designations, such as Orphan Drug, Fast Track and regenerative medicine advanced therapy, or RMAT, or such European equivalents, including PRIority MEdicines, or PRIME, designation;
- our ability to advance product candidates into, and successfully complete, clinical trials;
- our intellectual property coverage and positions, including those of our licensors and third parties as well as the status and potential outcome of proceedings involving any such intellectual property;
- our anticipated expenses, ability to obtain funding for our operations and the sufficiency of our cash resources; and
- the therapeutic value, development, and commercial potential of CRISPR/Cas9 gene editing technologies and therapies.

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and assumptions that could cause our actual results and the timing of certain events to differ materially from future results expressed or implied by the forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified herein, and those discussed in the section titled "Risk Factors," set forth in Part II, Item 1A of this Quarterly Report on Form 10-Q, if any, our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on February 21, 2023, and in other SEC filings. You should not rely upon forward-looking statements as predictions of future events. Such forward-looking statements speak only as of the date of this report. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make or enter into.

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, performance or achievements may be materially different from what we expect. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

Investors and others should note that we announce material information to our investors using our investor relations website (https://crisprtx.gcs-web.com/), SEC filings, press releases, public conference calls and webcasts. We use these channels as well as social media to communicate with the public about our Company, our business, our product candidates and other matters. It is possible that the information we post on social media could be deemed to be material information. Therefore, we encourage investors, the media, and others interested in our Company to review the information we post on the social media channels listed on our investor relations website.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements

CRISPR Therapeutics AG
Condensed Consolidated Balance Sheets
(unaudited, in thousands, except share and per share data)

	As of	
	March 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 344,407	$ 211,885
Marketable securities	1,538,763	1,603,433
Prepaid expenses and other current assets	24,796	37,708
Total current assets	1,907,966	1,853,026
Property and equipment, net	162,198	163,634
Marketable securities, non-current	6,320	53,130
Intangible assets, net	57	71
Restricted cash	11,717	11,635
Operating lease assets	153,346	156,921
Other non-current assets	2,760	4,640
Total assets	$ 2,244,364	$ 2,243,057
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 45,153	$ 27,428
Accrued expenses	85,631	77,682
Accrued tax liabilities	273	135
Operating lease liabilities	15,717	15,842
Other current liabilities	20	20
Total current liabilities	146,794	121,107
Deferred revenue, non-current	12,323	12,323
Operating lease liabilities, net of current portion	224,552	228,179
Other non-current liabilities	5,799	5,969
Total liabilities	389,468	367,578
Commitments and contingencies, see Note 7		
Shareholders' equity:		
Common shares, CHF 0.03 par value, 150,347,467 shares authorized at March 31, 2023 and at December 31, 2022, 79,044,050 and 78,692,766 shares issued at March 31, 2023 and December 31, 2022, respectively, 78,863,734 and 78,512,450 shares outstanding at March 31, 2023 and December 31, 2022, respectively	2,452	2,441
Treasury shares, at cost, 180,316 shares at March 31, 2023 and at December 31, 2022	(63)	(63)
Additional paid-in capital	2,761,050	2,734,838
Accumulated deficit	(899,155)	(846,090)
Accumulated other comprehensive loss	(9,388)	(15,647)
Total shareholders' equity	1,854,896	1,875,479
Total liabilities and shareholders' equity	$ 2,244,364	$ 2,243,057

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

CRISPR Therapeutics AG
Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited, in thousands, except share and per share data)

	Three Months Ended March 31,	
	2023	**2022**
Revenue:		
Collaboration revenue	$ 100,000	$ 178
Grant revenue	—	762
Total revenue	100,000	940
Operating expenses:		
Research and development	99,935	118,245
General and administrative	22,360	28,021
Collaboration expense, net	42,192	30,646
Total operating expenses	164,487	176,912
Loss from operations	(64,487)	(175,972)
Other income:		
Other income, net	12,742	363
Total other income, net	12,742	363
Net loss before income taxes	(51,745)	(175,609)
Provision for income taxes	(1,320)	(3,608)
Net loss	(53,065)	(179,217)
Foreign currency translation adjustment	32	(27)
Unrealized gain (loss) on marketable securities	6,227	(11,799)
Comprehensive loss	$ (46,806)	$ (191,043)
Net loss per common share — basic	$ (0.67)	$ (2.32)
Basic weighted-average common shares outstanding	78,676,986	77,098,319
Net loss per common share — diluted	$ (0.67)	$ (2.32)
Diluted weighted-average common shares outstanding	78,676,986	77,098,319

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

CRISPR Therapeutics AG
Condensed Consolidated Statements of Shareholders' Equity
(unaudited, in thousands, except share and per share data)

	Common Shares		Treasury Shares					
	Shares	CHF 0.03 Par Value	Shares	Amount, at cost	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total Shareholders' Equity
Balance at December 31, 2021	76,990,066	$ 2,391	180,316	$ (63)	$ 2,598,114	$ (195,915)	$ (5,067)	$ 2,399,460
Vesting of restricted shares	123,564	4	—	—	—	—	—	4
Exercise of vested options, net of issuance costs of $0.2 million	261,280	12	—	—	9,998	—	—	10,010
Purchase of common stock under ESPP	11,495	—	—	—	740	—	—	740
Stock-based compensation expense	—	—	—	—	25,745	—	—	25,745
Other comprehensive loss	—	—	—	—	—	—	(11,826)	(11,826)
Net loss	—	—	—	—	—	(179,217)	—	(179,217)
Balance at March 31, 2022	77,386,405	$ 2,407	180,316	$ (63)	$ 2,634,597	$ (375,132)	$ (16,893)	$ 2,244,916
Balance at December 31, 2022	78,512,450	$ 2,441	180,316	$ (63)	$ 2,734,838	$ (846,090)	$ (15,647)	$ 1,875,479
Vesting of restricted shares	172,995	5	—	—	—	—	—	5
Exercise of vested options, net of issuance costs of $0.2 million	159,184	6	—	—	4,677	—	—	4,683
Purchase of common stock under ESPP	19,105	—	—	—	660	—	—	660
Stock-based compensation expense	—	—	—	—	20,875	—	—	20,875
Other comprehensive income	—	—	—	—	—	—	6,259	6,259
Net loss	—	—	—	—	—	(53,065)	—	(53,065)
Balance at March 31, 2023	78,863,734	$ 2,452	180,316	$ (63)	$ 2,761,050	$ (899,155)	$ (9,388)	$ 1,854,896

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

CRISPR Therapeutics AG
Condensed Consolidated Statements of Cash Flows
(unaudited, in thousands)

	Three Months Ended March 31,	
	2023	**2022**
Operating activities:		
Net loss	$ (53,065)	$ (179,217)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation and amortization	5,051	6,039
Equity-based compensation	20,875	25,745
Other non-cash items, net	(969)	6,099
Changes in:		
Accounts receivable	—	162
Prepaid expenses and other assets	12,040	4,611
Accounts payable and accrued expenses	25,214	(1,616)
Deferred revenue	—	(762)
Operating lease assets and liabilities	(177)	3,854
Other liabilities, net	(170)	(154)
Net cash provided by (used in) operating activities	8,799	(135,239)
Investing activities:		
Purchase of property, plant and equipment	(3,059)	(15,350)
Purchases of marketable securities	(265,627)	(323,140)
Maturities of marketable securities	386,515	223,975
Net cash provided by (used in) investing activities	117,829	(114,515)
Financing activities:		
Proceeds from exercise of options and ESPP contributions, net of issuance costs	5,404	10,649
Net cash provided by financing activities	5,404	10,649
Effect of exchange rate changes on cash	32	(27)
Increase (decrease) in cash	132,064	(239,132)
Cash, cash equivalents and restricted cash, beginning of period	224,060	939,944
Cash, cash equivalents and restricted cash, end of period	$ 356,124	$ 700,812
Supplemental disclosure of non-cash investing and financing activities		
Property and equipment purchases in accounts payable and accrued expenses	$ 2,663	$ 5,888
Equity issuance costs in accounts payable and accrued expenses	$ 155	$ 217
Leasehold improvements paid directly by landlord	$ —	$ 13,015

	As of March 31,	
Reconciliation to amounts within the condensed consolidated balance sheets	**2023**	**2022**
Cash and cash equivalents	344,407	683,906
Prepaid expenses and other current assets	—	4,783
Restricted cash	11,717	12,123
Cash, cash equivalents and restricted cash at end of period	$ 356,124	$ 700,812

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

1. Basis of Presentation and Significant Accounting Policies

Basis of Presentation

The accompanying condensed consolidated financial statements are unaudited and have been prepared by the Company in accordance with accounting principles generally accepted in the United States of America, or U.S. GAAP.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. The Company views its operations and manages its business in one operating segment, which is the business of discovering, developing and commercializing therapies derived from or incorporating genome-editing technology. Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of operations for the three-month interim periods ended March 31, 2023 and 2022.

Beginning in 2022, "collaboration expense, net" in the condensed consolidated statements of operations and comprehensive loss present collaboration costs related to the exagamglogene autotemcel, or exa-cel (formerly known as CTX001), program under the Company's collaboration with Vertex Pharmaceuticals Incorporated and certain of its subsidiaries, or Vertex, accounted for under ASC 808, *Collaborative Agreements*, or ASC 808. Refer to Note 6 to these condensed consolidated financial statements for further discussion on the Company's agreements with Vertex, including the exa-cel program.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These interim financial statements should be read in conjunction with the audited financial statements for the year ended December 31, 2022, which are contained in the 2022 Annual Report on Form 10-K filed with the Securities and Exchange Commission, or the SEC, on February 21, 2023.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, revenue recognition, equity-based compensation expense and reported amounts of expenses during the period. Significant estimates in these consolidated financial statements have been made in connection with revenue recognition and equity-based compensation expense. The Company bases its estimates on historical experience and other market-specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions. Changes in estimates are reflected in reported results in the period in which they become known.

Significant Accounting Policies

The significant accounting policies used in preparation of these condensed consolidated financial statements for the three months ended March 31, 2023 are consistent with those discussed in Note 2 to the consolidated financial statements in the Company's 2022 Annual Report on Form 10-K filed with the SEC on February 21, 2023.

New Accounting Pronouncements – Recently Adopted

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board, or FASB, or other standard setting bodies that the Company adopts as of the specified effective date. The Company does not believe that the adoption of recently issued standards have or may have a material impact on its consolidated financial statements and disclosures.

2. Marketable Securities

The following table summarizes cash equivalents and marketable securities held at March 31, 2023 and December 31, 2022 (in thousands), which are recorded at fair value. The table below excludes $263.6 million and $159.3 million of cash at March 31, 2023 and December 31, 2022, respectively.

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
March 31, 2023				
Cash equivalents:				
Money market funds	$ 19,387	$ —	$ —	$ 19,387
Corporate debt securities	2,358	—	—	2,358
Commercial paper	13,527	—	(1)	13,526
U.S. Treasury securities	45,557	9	—	45,566
Total cash equivalents	80,829	9	(1)	80,837
Marketable securities:				
U.S. Treasury securities	2,991	—	(7)	2,984
Corporate debt securities	1,034,507	1,143	(9,773)	1,025,877
Certificates of deposit	81,948	—	—	81,948
Government-sponsored enterprise securities	145,034	81	(477)	144,638
Commercial paper	289,987	6	(357)	289,636
Total marketable securities	1,554,467	1,230	(10,614)	1,545,083
Total cash equivalents and marketable securities	$ 1,635,296	$ 1,239	$ (10,615)	$ 1,625,920
December 31, 2022				
Cash equivalents:				
Money market funds	$ 17,766	$ —	$ —	$ 17,766
Corporate debt securities	2,151	—	(2)	2,149
Commercial paper	32,675	—	—	32,675
Total cash equivalents	52,592	—	(2)	52,590
Marketable securities:				
Corporate debt securities	1,236,770	615	(15,006)	1,222,379
Certificates of deposit	92,417	—	—	92,417
Government-sponsored enterprise securities	79,746	11	(712)	79,045
Commercial paper	263,231	—	(509)	262,722
Total marketable securities	1,672,164	626	(16,227)	1,656,563
Total cash equivalents and marketable securities	$ 1,724,756	$ 626	$ (16,229)	$ 1,709,153

As of March 31, 2023 and December 31, 2022, marketable securities were in a net unrealized loss position of $9.4 million and $15.6 million, respectively. The Company has recorded a net unrealized gain of $6.2 million and a net unrealized loss of $11.8 million during the three months ended March 31, 2023 and 2022, respectively, related to its debt securities, which is included in comprehensive loss on the condensed consolidated statements of operations and comprehensive loss.

As of March 31, 2023 and December 31, 2022, the aggregate fair value of marketable securities that were in an unrealized loss position for less than twelve months was $630.5 million and $628.4 million, respectively. As of March 31, 2023 and December 31, 2022, the aggregate fair value of marketable securities that were in an unrealized loss position for more than twelve months was $468.0 million and $619.2 million, respectively. Of this amount, securities totaling $6.3 million and $53.1 million as of March 31, 2023 and December 31, 2022, respectively, will mature beyond one year.

The Company determined that there is no material credit risk associated with the above investments as of March 31, 2023. The Company has the intent and ability to hold such securities until recovery. As a result, the Company did not record any charges for credit-related impairments for its marketable securities for the three months ended March 31, 2023 and 2022. No available-for-sale debt securities held as of March 31, 2023 had remaining maturities greater than thirty months.

3. Fair Value Measurements

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the fair value hierarchy classification of such fair values as of March 31, 2023 and December 31, 2022 (in thousands):

	Fair Value Measurements at March 31, 2023			
	Total	Level 1	Level 2	Level 3
Cash and cash equivalents:				
Cash	$ 263,570	$ 263,570	$ —	$ —
Money market funds	19,387	19,387	—	—
Corporate debt securities	2,358	—	2,358	—
Commercial paper	13,526	—	13,526	—
U.S. Treasury securities	45,566	—	45,566	—
Marketable securities:				
U.S. Treasury securities	2,984	—	2,984	—
Corporate debt securities	1,025,877	—	1,025,877	—
Certificates of deposit	81,948	—	81,948	—
Government-sponsored enterprise securities	144,638	—	144,638	—
Commercial paper	289,636	—	289,636	—
Total	$ 1,889,490	$ 282,957	$ 1,606,533	$ —

	Fair Value Measurements at December 31, 2022			
	Total	Level 1	Level 2	Level 3
Cash and cash equivalents:				
Cash	$ 159,295	$ 159,295	$ —	$ —
Money market funds	17,766	17,766	—	—
Corporate debt securities	2,149	—	2,149	—
Commercial paper	32,675	—	32,675	—
Marketable securities:				
Corporate debt securities	1,222,379	—	1,222,379	—
Certificates of deposit	92,417	—	92,417	—
Government-sponsored enterprise securities	79,045	—	79,045	—
Commercial paper	262,722	—	262,722	—
Other non-current assets	2,212	—	—	2,212
Total	$ 1,870,660	$ 177,061	$ 1,691,387	$ 2,212

Marketable securities classified as Level 2 within the valuation hierarchy generally consist of U.S. treasury securities and government agency securities, certificates of deposit, corporate bonds and commercial paper. The Company estimates the fair value of these marketable securities by taking into consideration valuations obtained from third-party pricing sources.

4. Property and Equipment, net

Property and equipment, net, consists of the following (in thousands):

	As of	
	March 31, 2023	December 31, 2022
Computer equipment	$ 3,618	$ 3,618
Furniture, fixtures and other	8,109	8,109
Laboratory equipment	40,176	37,897
Leasehold improvements	143,060	141,680
Construction work in process	6,104	6,162
Total property and equipment, gross	201,067	197,466
Accumulated depreciation	(38,869)	(33,832)
Total property and equipment, net	$ 162,198	$ 163,634

Depreciation expense for the three months ended March 31, 2023 and 2022 was $5.0 million and $6.0 million, respectively.

5. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	As of	
	March 31, 2023	December 31, 2022
Payroll and employee-related costs	$ 8,351	$ 19,241
Research costs	22,354	35,010
Collaboration costs	44,824	11,177
Licensing fees	1,068	983
Professional fees	4,041	4,927
Intellectual property costs	2,985	3,936
Accrued property and equipment	949	1,244
Other	1,059	1,164
Total	$ 85,631	$ 77,682

6. Significant Contracts

Agreements with Vertex

2015 collaboration

In 2015, the Company entered into a strategic collaboration, option and license agreement, or the 2015 Collaboration Agreement, with Vertex. The 2015 Collaboration Agreement is focused on the use of the Company's CRISPR/Cas9 gene editing technology to discover and develop potential new treatments aimed at the underlying genetic causes of human disease. The Company and Vertex amended the 2015 Collaboration Agreement in 2017 and 2019 with Amendment No. 1 and Amendment No. 2, respectively, namely to clarify Vertex's option rights under the 2015 Collaboration Agreement and to modify certain definitions and provisions of the 2015 Collaboration Agreement to make them consistent with the JDA (as defined below) and the 2019 Collaboration Agreement (as defined below). In 2017, Vertex exercised an option granted to it under the 2015 Collaboration Agreement to obtain a co-exclusive license to develop and commercialize hemoglobinopathy and beta-globin targets, and in 2019, Vertex exercised the remaining options granted to it under the 2015 Collaboration Agreement to exclusively license certain collaboration targets developed under the 2015 Collaboration Agreement.

Exa-cel collaboration

In 2017, following Vertex's exercise of its option to obtain a co-exclusive license to develop and commercialize hemoglobinopathy and beta-globin targets, the Company and Vertex entered into a joint development and commercialization agreement, or the JDA, and agreed for potential hemoglobinopathy treatments, including exa-cel, the Company and Vertex would share equally all research and development costs and worldwide revenues. In 2021, the Company and Vertex amended and restated the JDA, or the A&R Vertex JDCA, pursuant to which the parties agreed to, among other things, (a) adjust the governance structure for the collaboration and adjust the responsibilities of each party thereunder, whereby Vertex leads and has all decision making (i.e., control) in relation to the exa-cel program prospectively; (b) adjust the allocation of net profits and net losses between the parties with respect to exa-cel only, which will be allocated 40% to the Company and 60% to Vertex, prospectively; and (c) exclusively license (subject to the Company's reserved rights to conduct certain activities) certain intellectual property rights to Vertex relating to the specified product candidates and products (including exa-cel) that may be researched, developed, manufactured and commercialized on a worldwide basis under the A&R Vertex JDCA. Additionally, the A&R Vertex JDCA allows the Company to defer a portion of its share of costs under the arrangement if spending on the exa-cel program exceeds specified amounts. Any deferred amounts are only payable to Vertex as an offset against future profitability of the exa-cel program and the amounts payable are capped at a specified maximum amount per year.

DMD and DM1 exclusive license

In 2019, the Company and Vertex entered into a series of agreements, including a strategic collaboration and license agreement, or the 2019 Collaboration Agreement, for the development and commercialization of products for the treatment of Duchenne muscular dystrophy, or DMD, and Myotonic Dystrophy Type 1, or DM1. For the DMD and DM1 programs, Vertex is responsible for all research, development, manufacturing and commercialization activities and all related costs. Upon IND filing, the Company has the option to forego the DM1 milestones and royalties, and instead, co-develop and co-commercialize all DM1 products globally in

exchange for payment of 50% of research and development costs incurred by Vertex from the effective date of the agreement through IND filing.

Collaboration in the field of diabetes

In 2021, CRISPR and ViaCyte, Inc., or ViaCyte, entered into a joint development and commercialization agreement, or the ViaCyte JDCA, to jointly develop and commercialize product candidates and shared products for the diagnosis, treatment or prevention of diabetes type 1, diabetes type 2 or insulin dependent / requiring diabetes throughout the world. In the third quarter of 2022, Vertex acquired ViaCyte, and ViaCyte became a wholly-owned subsidiary of Vertex. In March 2023, (1) the Company and ViaCyte entered into an amendment to the ViaCyte JDCA, or the ViaCyte JDCA Amendment, and adjusted certain rights and obligations of the Company and ViaCyte under the ViaCyte JDCA, and (2) the Company and Vertex entered into a non-exclusive license agreement, or the Non-Ex License Agreement, pursuant to which the Company agreed to license to Vertex, on a non-exclusive basis, certain of its gene editing intellectual property to exploit certain products for the diagnosis, treatment or prevention of diabetes type 1, diabetes type 2 or insulin dependent / requiring diabetes throughout the world. In connection with entering into these agreements, the Company received a $100.0 million up front payment from Vertex. Under the Non-Ex License Agreement, the Company is eligible to receive milestone payments from Vertex of up to $230.0 million in the aggregate, depending on the achievement of pre-determined research, development and commercial milestones for certain products utilizing the licensed intellectual property. Additionally, the Company is eligible to receive tiered royalties on the sales of certain products in the low to mid-single digits.

Accounting Analysis

The 2015 Collaboration Agreement, Amendment No. 1, Amendment No. 2, A&R Vertex JDCA, and 2019 Collaboration Agreement are collectively the “Vertex Agreements” for purposes of this Note 6. The Non-Ex License Agreement and ViaCyte JDCA Amendment are collectively the “March 2023 Agreements.”

The Vertex Agreements and the March 2023 Agreements include components of a customer-vendor relationship as defined under ASC 606, *Revenue from Contracts with Customers*, or ASC 606, collaborative arrangements as defined under ASC 808, *Collaborative Agreements*, or ASC 808, and research and development costs as defined under ASC 730, *Research and Development*, or ASC 730. Specifically, with regards to the March 2023 Agreements, the Company concluded that the non-exclusive license is a performance obligation under ASC 606 and the ongoing research and development services under the ViaCyte JDCA Amendment are a unit of account under ASC 808.

Accounting Analysis Under ASC 606

March 2023 Agreements

Identification of the contract

The March 2023 Agreements were negotiated as a package with a single commercial objective and, as such, the March 2023 Agreements were combined for accounting purposes and treated as a single arrangement. The Company determined for accounting purposes that the combined contract terminates the original ViaCyte JDCA and created a new contract.

Identification of performance obligations

The Company concluded the transfer of the non-exclusive license, including certain modified rights and obligations provided as part of the ViaCyte JDCA Amendment to support the delivery of the license, was both capable of being distinct and distinct within the context of the contract.

Determination of transaction price

The transaction price was comprised of the upfront payment of $100.0 million. The Company determined that all other possible variable consideration resulting from milestones and royalties discussed below was fully constrained at the time of the transaction. The Company will reevaluate the transaction price in each reporting period.

Allocation of transaction price to performance obligations

The Company identified one performance obligation for the March 2023 Agreements and, as a result, no allocation of the transaction price was required.

Recognition of revenue

The Company determined the non-exclusive license, including certain modified rights and obligations provided as part of the ViaCyte JDCA Amendment to support the delivery of the license, represented functional intellectual property, as the intellectual property provides Vertex with the ability to perform a function or task in the form of research and development in the field of diabetes. The Company recognized revenue for the non-exclusive license at the onset of the arrangement, as this was the point in time in which the non-exclusive license was delivered. Revenue recognized under the March 2023 Agreements was $100.0 million for the three months ended March 31, 2023.

Milestones under the Non-Ex License Agreement

The Company is eligible to receive milestone payments from Vertex of up to $230.0 million in the aggregate under the Non-Ex License Agreement, depending on the achievement of pre-determined research, development and commercial milestones for certain products utilizing the licensed intellectual property. Additionally, the Company is eligible to receive tiered royalties on the sales of certain products in the low to mid-single digits. Each of the milestones under the Non-Ex License Agreement are fully constrained as of March 31, 2023. There is uncertainty as to whether the events to obtain the research and developmental milestones will be achieved given the nature of clinical development and the stage of the CRISPR/Cas9 technology. The remaining research, development and regulatory milestones will be constrained until it is probable that a significant revenue reversal will not occur. Commercial milestones and royalties relate predominantly to a license of intellectual property and are determined by sales or usage-based thresholds. The commercial milestones and royalties are accounted for under the royalty recognition constraint and will be accounted for as constrained variable consideration. The Company applies the royalty recognition constraint for each commercial milestone and will not recognize revenue for each until the subsequent sale of a licensed product (achievement of each) occurs.

Vertex Agreements

Deferred revenue

As of March 31, 2023 and December 31, 2022, there was no current deferred revenue related to the Vertex Agreements. As of March 31, 2023, there was $12.3 million of non-current deferred revenue related to the Vertex Agreements, which is unchanged from December 31, 2022. The transaction price allocated to the remaining performance obligations was $12.3 million.

Milestones

The Company has evaluated the milestones that may be received in connection with the Vertex Agreements.

Under the 2015 Collaboration Agreement and subsequent amendments, the Company is eligible to receive up to $410.0 million in additional development, regulatory and commercial milestones and royalties on net product sales for each of the three collaboration targets that Vertex licensed in 2019. Each milestone is payable only once per collaboration target, regardless of the number of products directed to such collaboration target that achieve the relevant milestone event.

The Company is eligible to receive potential future payments of up to $775.0 million under the 2019 Collaboration Agreement based upon the successful achievement of specified development, regulatory and commercial milestones for the DMD and DM1 programs. The Company is also eligible to receive tiered royalties on future net sales on any products that may result from this collaboration; however, the Company has the option to forego the DM1 milestones and royalties to co-develop and co-commercialize all DM1 products globally.

The Company is eligible to receive potential future payments of up to $200.0 million under the A&R Vertex JDCA upon receipt by Vertex of the first marketing approval of the initial product candidate from the FDA or the European Commission. In addition, the Company has the option to conduct research at their own cost in certain defined areas that, if beneficial to the exa-cel program and exa-cel ultimately achieves regulatory approval in such areas, then the Company could be entitled to certain milestone payments aggregating to high eight digits from Vertex.

Each of the remaining milestones described above are fully constrained as of March 31, 2023. There is uncertainty that the events to obtain the research and developmental milestones will be achieved given the nature of clinical development and the stage of the CRISPR/Cas9 technology. The remaining research, development and regulatory milestones will be constrained until it is probable that a significant revenue reversal will not occur. Commercial milestones and royalties relate predominantly to a license of intellectual property and are determined by sales or usage-based thresholds. The commercial milestones and royalties are accounted for under the royalty recognition constraint and will be accounted for as constrained variable consideration. The Company applies the royalty recognition constraint for each commercial milestone and will not recognize revenue for each until the subsequent sale of a licensed product (achievement of each) occurs.

Accounting Analysis under ASC 808

Vertex Agreements

In connection with the Vertex Agreements, the Company identified the following collaborative elements, which are accounted for under ASC 808: (i) development and commercialization services for shared products, including any transition services related to exa-cel under the A&R Vertex JDCA; (ii) R&D Services for follow-on products; and (iii) committee participation. The related impact of the cost sharing is included within collaboration expense, net, in the condensed consolidated statements of operations and comprehensive loss. During the three months ended March 31, 2023 and 2022, the Company recognized $42.2 million and $30.6 million of collaboration expense, net, related to the exa-cel program, respectively. Collaboration expense, net, was net of $2.8 million and $7.4 million of reimbursements from Vertex related to the exa-cel program, respectively.

7. Commitments and Contingencies

Leases

Refer to Note 7 to the consolidated financial statements in the Company's 2022 Annual Report on Form 10-K filed with the SEC on February 21, 2023 for discussion of the Company's lease arrangements.

Litigation

In the ordinary course of business, the Company is from time to time involved in lawsuits, investigations, proceedings and threats of litigation related to, among other things, the Company's intellectual property estate (including certain in-licensed intellectual property), commercial arrangements and other matters. Such proceedings may include quasi-litigation, *inter partes* administrative proceedings in the U.S. Patent and Trademark Office and the European Patent Office involving the Company's intellectual property estate including certain in-licensed intellectual property. The outcome of any of the foregoing, regardless of the merits, is inherently uncertain. In addition, litigation and related matters are costly and may divert the attention of Company's management and other resources that would otherwise be engaged in other activities. If the Company is unable to prevail in any such proceedings, the Company's business, results of operations, liquidity and financial condition could be adversely affected.

Letters of Credit

As of March 31, 2023, the Company had restricted cash of $11.7 million, representing letters of credit securing the Company's obligations under certain leased facilities. The letters of credit are secured by cash held in a restricted depository account and included in "Restricted cash" on the Company's condensed consolidated balance sheets as of March 31, 2023.

Research, Manufacturing, License and Intellectual Property Agreements

The Company has engaged several research institutions and companies to identify new delivery strategies and applications of the Company's gene editing technology. The Company is also a party to a number of license agreements which require significant upfront payments and may be required to make future royalty payments and potential milestone payments from time to time. In addition, the Company is also a party to intellectual property agreements, which require maintenance and milestone payments from time to time. Further, the Company is a party to a number of manufacturing agreements that require upfront payments for the future performance of services.

In association with these agreements, on a product-by-product basis, the counterparties are eligible to receive up to low eight-digit potential payments upon specified research, development and regulatory milestones. In addition, on a product-by-product basis, the counterparties are eligible to receive potential commercial milestone payments based on specified annual sales thresholds. The potential payments are low-single digit percentages of the specified annual sales thresholds. The counterparties are also eligible to receive low single-digit royalties on future net sales.

Under certain circumstances and if certain contingent future events occur, Vertex is eligible to receive up to $395.0 million in potential specified research, development, regulatory and commercial milestones and tiered single-digit percentage royalties on future net sales related to a specified target under an amendment to the 2015 Collaboration Agreement (as such term is defined in Note 6 above). In addition, Vertex has the option to conduct research at its own cost in certain defined areas that, if beneficial to the exa-cel program and ultimately achieves regulatory approval, could result in the Company owing Vertex certain milestone payments aggregating to high eight digits, subject to certain limitations on the profitability of the exa-cel program.

Under the A&R Vertex JDCA, the Company deferred $36.1 million of its share of costs incurred under the arrangement for the year ended December 31, 2022, as spending on the exa-cel program exceeded a specified amount. Any deferred amounts are only payable to Vertex as an offset against future profitability of the exa-cel program and the amounts payable are capped at a specified maximum amount per year. These deferred costs on the exa-cel program will be accrued for when it is probable that a liability has been incurred and the amount can be reasonably estimated. As of March 31, 2023, no contingent payments have been accrued. Refer to Note 6 for further discussion of the Company's arrangements with Vertex.

8. Share Capital

The Company had 150,347,467 authorized common shares as of March 31, 2023, with a par value of CHF 0.03 per share. Share Capital consisted of the following:

		As of	
Type of Share Capital	**Conditional Capital**	**March 31, 2023**	**December 31, 2022**
Common shares	Registered share capital	83,538,347	82,028,328
Common shares	Authorized share capital	39,316,975	39,316,975
Common shares	Conditional share capital - Bonds or similar debt instruments	8,202,832	8,202,832
Common shares	Conditional share capital - Employee benefit plans	19,289,313	20,799,332
	Total	150,347,467	150,347,467

Common Share Issuances

At-the-Market Offering

In August 2019, the Company entered into an Open Market Sale Agreement[SM] with Jefferies LLC, or Jefferies, under which the Company was able to offer and sell, from time to time at its sole discretion through Jefferies, as its sales agent, its common shares, or the August 2019 Sales Agreement.

In January 2021, in connection with the August 2019 Sales Agreement, the Company filed a prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to $600.0 million. In July 2021, the Company filed a new prospectus supplement with the SEC, which replaced the previous prospectus supplement filed in January 2021, to offer and sell, from time to time, the common shares remaining under the original prospectus supplement having aggregate gross proceeds of up to $419.8 million, or, together with the January 2021 prospectus supplement, the 2021 ATM.

As of March 31, 2023, the Company has issued and sold an aggregate of 1.1 million common shares under the 2021 ATM at an average price of $168.79 per share for aggregate proceeds of $178.8 million, which were net of equity issuance costs of $2.4 million.

9. Stock-based Compensation

During the three months ended March 31, 2023 and 2022, the Company recognized the following stock-based compensation expense (in thousands):

	Three Months Ended March 31,	
	2023	**2022**
Research and development	$ 11,676	$ 14,589
General and administrative	9,199	11,156
Total	$ 20,875	$ 25,745

Stock option activity

The following table summarizes stock option activity for the three months ended March 31, 2023:

	Shares	Weighted-average exercise price per share
Outstanding at December 31, 2022	7,230,233	$ 60.22
Granted	1,236,882	44.05
Exercised	(159,184)	30.37
Cancelled or forfeited	(390,947)	86.12
Outstanding at March 31, 2023	7,916,984	$ 57.01
Exercisable at March 31, 2023	4,853,602	$ 52.83
Vested and expected to vest at March 31, 2023	7,916,984	$ 57.01

As of March 31, 2023, total unrecognized compensation expense related to stock options was $117.4 million, which the Company expects to recognize over a remaining weighted-average period of 2.6 years.

Restricted stock activity

The following table summarizes restricted stock activity for the three months ended March 31, 2023:

	Shares	Weighted-Average Grant Date Fair Value
Unvested balance at December 31, 2022	1,325,185	$ 80.13
Granted	643,724	43.76
Vested	(172,995)	74.21
Cancelled or forfeited	(125,084)	86.11
Unvested balance at March 31, 2023	1,670,830	$ 66.29

As of March 31, 2023, total unrecognized compensation expense related to unvested restricted common shares was $82.0 million, which the Company expects to recognize over a remaining weighted-average vesting period of 2.8 years.

10. Net Loss Per Share Attributable to Common Shareholders

Basic net loss per share is calculated by dividing net loss attributable to common shareholders by the weighted-average number of common shares outstanding during the period. Diluted net loss per share is calculated by dividing the net loss attributable to common shareholders by the weighted-average number of common share equivalents outstanding for the period, including any dilutive effect from outstanding stock options and warrants using the treasury stock method. The Company's net loss is net loss attributable to common shareholders for all periods presented.

The following common stock equivalents were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect (in thousands):

	Three Months Ended March 31,	
	2023	2022
Outstanding options	7,916,984	8,220,458
Unvested restricted common shares	1,670,830	1,147,364
ESPP	18,110	10,969
Total	9,605,924	9,378,791

11. Income Taxes

During the three months ended March 31, 2023 and 2022, the Company recorded an income tax provision of $1.3 million and $3.6 million, respectively, representing an effective tax rate of (2.6%) and (2.1%), respectively. The income tax provision for the three months ended March 31, 2023 and 2022 is primarily attributable to the Company's U.S. subsidiaries. The change in the rate for the three months ended March 31, 2023 is primarily attributable to reduced forecasted capitalized R&D expense addback offset by an increase in forecasted interest income in the United States. The difference in the statutory tax rate and effective tax rate is primarily a result of the jurisdictional mix of earnings, research credits generated, and the valuation allowance recorded against certain deferred tax assets. The Company maintains a valuation allowance against certain deferred tax assets that are not more-likely-than-not realizable.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with (i) our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and (ii) our audited consolidated financial statements and related notes and management's discussion and analysis of financial condition and results of operations included in our Annual Report on Form 10-K for the year ended December 31, 2022 filed with the Securities and Exchange Commission, or the SEC, on February 21, 2023. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and impact and potential impacts on our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including, without limitation, those factors set forth in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2022 and the "Risk Factors" section of subsequent Quarterly Reports on Form 10-Q, our actual results or timing of certain events could differ materially from the results or timing described in, or implied by, these forward-looking statements.

Overview

We are a leading gene editing company focused on the development of CRISPR/Cas9-based therapeutics. CRISPR/Cas9 is a revolutionary gene editing technology that allows for precise, directed changes to genomic DNA. The application of CRISPR/Cas9 for gene editing was co-invented by one of our scientific founders, Dr. Emmanuelle Charpentier. Dr. Charpentier and her collaborators published work elucidating how CRISPR/Cas9, a naturally occurring viral defense mechanism found in bacteria, can be adapted for use in gene editing. We are applying this technology to disrupt, delete, correct and insert genes to potentially treat genetically defined diseases and engineer advanced cellular therapies. We believe that our scientific expertise, together with our gene editing approach, may enable an entirely new class of highly effective and potentially curative therapies for patients with both rare and common diseases for whom current biopharmaceutical approaches have had limited success.

We have established a portfolio of therapeutic programs in a broad range of disease areas across four core franchises: hemoglobinopathies, immuno-oncology, regenerative medicine and *in vivo* approaches. Our most advanced programs target the genetically defined diseases transfusion-dependent beta thalassemia, or TDT, and severe sickle cell disease, or SCD, two hemoglobinopathies with high unmet medical need. We are also progressing several gene-edited allogeneic cell therapy programs, including allogeneic chimeric antigen receptor T cell, or CAR T, candidates for the treatment of hematological and solid tumor cancers, and investigational, allogeneic, gene-edited, immune-evasive, stem cell-derived therapies for the treatment of type 1 diabetes, or T1D. In addition, we are advancing multiple programs leveraging *in vivo* editing approaches, initially for the treatment and prevention of cardiovascular disease.

Hemoglobinopathies

Our lead product candidate, exa-cel, is an investigational, autologous, *ex vivo* CRISPR gene-edited hematopoietic stem cell therapy that is being evaluated for the treatment of TDT and severe SCD. Exa-cel is being developed under a joint development and commercialization agreement between us and Vertex. We and Vertex are investigating exa-cel in two ongoing Phase 1/2/3 open-label clinical trials that are designed to assess the safety and efficacy of a single dose of exa-cel in patients ages 12 to 35 with TDT (CLIMB-111) or severe SCD (CLIMB-121), respectively. Enrollment is complete for both CLIMB-111 and CLIMB-121. In the second and fourth quarters of 2022, at the European Hematology Association Congress and American Society of Hematology Annual Meeting, respectively, we presented updated clinical data from CLIMB-111 and CLIMB-121 for 44 patients with TDT and 31 patients with severe SCD treated with exa-cel. In addition, we and Vertex are conducting two additional Phase 3 open-label clinical trials of exa-cel in pediatric patients with TDT (CLIMB-141) and severe SCD (CLIMB-151). Patients who received exa-cel in CLIMB-111, CLIMB-121, CLIMB-141 or CLIMB-151 are followed for approximately two years after exa-cel infusion and are asked to participate in a long-term, open-label follow-up trial, CLIMB-131, to evaluate the safety and efficacy of exa-cel. CLIMB-131 is designed to follow participants for up to 15 years after exa-cel infusion.

We and Vertex recently completed rolling submissions of the Biologics Licensing Applications, or BLAs, for exa-cel in the United States. Exa-cel has been granted RMAT, Fast Track, Orphan Drug, and Rare Pediatric Disease designations by the FDA for the treatment of both TDT and SCD. In addition, in the fourth quarter of 2022, we and Vertex completed regulatory submissions for exa-cel in the EU and the United Kingdom, and the European Medicines Agency, or EMA, and the Medicines and Healthcare products Regulatory Agency, or MHRA, have validated the marketing applications, respectively, indicating acceptance of the marketing applications and initiation of the review. Exa-cel has also been granted Orphan Drug Designation from the European Commission, as well as PRIME designation from the EMA for the treatment of both TDT and SCD. In the United Kingdom, exa-cel has been granted an Innovation Passport under the Innovative Licensing and Access Pathway from the MHRA.

In addition, building upon exa-cel, we have next-generation efforts in targeted conditioning and *in vivo* editing of hematopoietic stem cells, either of which could broaden the number of patients that can benefit from our therapies.

Immuno-Oncology

We believe CRISPR/Cas9 has the potential to create the next generation of CAR T cell therapies that may have a superior product profile compared to current autologous therapies and allow accessibility to broader patient populations. Drawing from the *ex vivo* gene editing capabilities gained through our lead programs, we are advancing several immuno-oncology cell therapy programs, including allogeneic CAR T programs targeting Cluster of Differentiation 19, or CD19, and Cluster of Differentiation 70, or CD70.

CD19 Franchise

CTX110, our lead immuno-oncology product candidate, is a healthy donor-derived gene-edited allogeneic CAR T investigational therapy targeting CD19. We are investigating CTX110 in our CARBON clinical trial, which is designed to assess the safety and efficacy of CTX110 in adult patients with relapsed or refractory CD19-positive B-cell malignancies who have received at least two prior lines of therapy. CTX110 has been granted RMAT designation by the FDA.

The Phase 1 CARBON clinical trial is being conducted in two parts – Part A and Part B. In Part A of the Phase 1 CARBON clinical trial, or Phase 1 Part A, patients were infused with a single dose of CTX110 across escalating dose levels following a standard lymphodepletion regimen, with an option to re-dose CTX110 based on clinical benefit. In Part B of the Phase 1 CARBON clinical trial, or Phase 1 Part B, patients received CTX110 at Dose Level 4 following standard lymphodepletion, as well as a consolidation dose of CTX110 at the same dose level between four and eight weeks after the initial dose for patients that demonstrated clinical benefit.

In the fourth quarter of 2022, we presented updated clinical data from Phase 1 Part A for 32 patients treated with CTX110, which showed the potential for CTX110 to achieve long-term durable complete remissions with a positively differentiated safety profile in heavily pre-treated patients, and described emerging data from Phase 1 Part B, which showed an encouraging efficacy profile with the potential to improve efficacy with the use of a consolidation dose. Based on this emerging data from our Phase 1 CARBON clinical trial and discussions with regulatory agencies, we have expanded CARBON to include a Phase 2, potentially registrational, single-arm, multi-center, open-label clinical trial that incorporates consolidation dosing. The Phase 2 clinical trial is ongoing.

In parallel with CTX110, we are advancing CTX112, a next-generation investigational, allogeneic CAR T product candidate targeting CD19. CTX112 incorporates additional edits designed to enhance CAR T potency and reduce CAR T exhaustion. In the fourth quarter of 2022, the IND for CTX112 was cleared by the FDA. CTX112 is being investigated in an ongoing Phase 1/2 clinical trial designed to assess the safety and efficacy of CTX112 in adult patients with relapsed or refractory CD19-positive B-cell malignancies who have received at least two prior lines of therapy.

CD70 Franchise

CTX130 is a healthy donor-derived gene-edited allogeneic CAR T investigational therapy targeting CD70, an antigen expressed on various solid tumors and hematologic malignancies. CTX130 is being investigated in two ongoing independent Phase 1, single-arm, multi-center, open-label clinical trials, COBALT, that are designed to assess the safety and efficacy of several dose levels of CTX130 in adult patients. The COBALT-LYM trial is evaluating the safety and efficacy of CTX130 for the treatment of relapsed or refractory T or B cell malignancies. The COBALT-RCC trial is evaluating the safety and efficacy of CTX130 for the treatment of relapsed or refractory clear cell renal cell carcinoma. CTX130 has received Orphan Drug Designation from the FDA for the treatment of T cell lymphoma and RMAT designation for the treatment of Mycosis Fungoides and Sézary Syndrome (MF/SS), subtypes of Cutaneous T cell Lymphoma (CTCL). In the second quarter of 2022, at the European Hematology Association Congress, we released initial clinical data from the ongoing COBALT-LYM trial for 18 patients with T cell lymphoma treated with CTX130 who had reached at least 28 days of follow-up. Also, in the fourth quarter of 2022, at the Society of Immuno-therapy in Cancer Annual Meeting, we released initial clinical data from the COBALT-RCC trial for 14 patients.

In parallel with CTX130, we are advancing CTX131, a next-generation investigational allogeneic CAR T product candidate targeting CD70 in a basket of solid tumors. CTX131 incorporates additional edits designed to enhance CAR T potency and reduce CAR T exhaustion. In the first quarter of 2023, the IND for CTX131 was cleared by the FDA. CTX131 is being investigated in an ongoing Phase 1/2 clinical trial designed to assess the safety and efficacy of CTX131 in adult patients with relapsed or refractory solid tumors.

Additional candidates

Our CRISPR/Cas9 platform enables us to innovate continuously by incorporating incremental edits into next-generation products. In addition to CTX112 and CTX131, we are advancing several additional investigational CAR T product candidates.

Regenerative Medicine

Regenerative medicine, or the use of stem cells to repair or replace tissue or organ function lost due to disease, damage or age, holds the potential to treat both rare and common diseases. Building upon our *ex vivo* gene editing expertise, we have expanded our efforts in this field with a focus on allogeneic stem cell-derived therapies gene edited using CRISPR/Cas9 to enable immune evasion,

improve cell function, and direct cell fate. Our first major effort in this area is in diabetes, and we and ViaCyte, Inc., or ViaCyte, which was acquired by Vertex in the third quarter of 2022, are advancing a series of programs as part of a strategic collaboration for the discovery, development, and commercialization of gene-edited stem cell therapies for the treatment of diabetes. We believe the combination of ViaCyte's stem cell capabilities and our gene editing capabilities has the potential to enable a beta-cell replacement product candidate that may deliver durable benefit to patients without requiring concurrent immune suppression.

We have a multi-staged product strategy that leverages our CRISPR/Cas9 platform to advance multiple product candidates incorporating incremental edits designed to increase benefit. Our initial product candidate, VCTX210, is an investigational, allogeneic, gene-edited, immune-evasive, stem cell-derived product candidate for the treatment of type 1 diabetes, or T1D, developed by applying our gene editing technology to ViaCyte's proprietary stem cell capabilities. VCTX210 has gene edits designed to promote immune evasion and cell fitness. We and ViaCyte are investigating VCTX210 in an ongoing Phase 1 clinical trial that is designed to assess VCTX210's safety, tolerability, and immune evasion in patients with T1D, and we are in the follow-up stage for this clinical trial. Our next generation product candidate, VCTX211, is an investigational, allogeneic, gene-edited, stem cell-derived product candidate for the treatment of T1D, which incorporates additional gene edits that aim to further enhance cell fitness. In the fourth quarter of 2022, the Clinical Trial Application for VCTX211 was cleared by Health Canada. VCTX211 is being investigated in an ongoing Phase 1/2 clinical trial designed to assess the safety, tolerability and efficacy of VCTX211 in adult patients with T1D.

In Vivo

Our *in vivo* gene editing strategy focuses on gene disruption and whole gene correction – the two technologies required to address the vast majority of the most prevalent severe monogenic diseases. We have established a leading platform for *in vivo* gene disruption in the liver and are rapidly advancing a broad portfolio of *in vivo* programs for both rare and common diseases towards clinical trials. Within the liver, we are pursuing diseases that are amenable to a gene disruption strategy and have well-understood genetic linkages, such as cardiovascular disease, or CVD. Our lead investigational *in vivo* programs, CTX310 and CTX320, target angiopoietin-related protein 3 (ANGPTL3) and lipoprotein(a) (Lp(a)), respectively, two validated targets for CVD. We believe this approach of leveraging existing proofs of concept reduces the challenges associated with delivering CRISPR/Cas9-based therapeutics *in vivo*. Beyond the liver, for delivery to hematopoietic stem cells, the central nervous system, and other extrahepatic tissues, we are pursuing additional delivery technologies, including adeno-associated virus vectors, or AAV, and further advancements to nanoparticle technology.

CRISPR-X

While we have made significant progress with our current portfolio of programs, we recognize that we need to continue to innovate to unlock the full potential of CRISPR gene editing and bring the potential of transformative therapies to even more patients. In 2022, we launched a new early-stage research team known as CRISPR-X that focuses on innovative research to develop next-generation editing modalities. CRISPR-X focuses on technologies to enable whole gene correction and insertion without requiring homology-directed repair or viral delivery of DNA, such as all-RNA gene correction, non-viral delivery of DNA and novel gene insertion techniques.

Partnerships

Given the numerous potential therapeutic applications for CRISPR/Cas9, we have partnered strategically to broaden the indications we can pursue and accelerate development of programs by accessing specific technologies and/or disease-area expertise. We maintain broad partnerships to develop gene editing-based therapeutics in specific disease areas.

Vertex. We established our initial collaboration agreement in 2015 with Vertex, which focused on TDT, SCD, cystic fibrosis and select additional indications. In December 2017, we entered into a joint development and commercialization agreement with Vertex pursuant to which, among other things, we are co-developing and preparing to co-commercialize exa-cel for TDT and SCD. In April 2021, we and Vertex amended and restated our existing joint development and commercialization agreement, pursuant to which, among other things, we will continue to develop and prepare to commercialize exa-cel for TDT and severe SCD in partnership with Vertex. In addition, we entered into a strategic collaboration and license agreement with Vertex in June 2019 for the development and commercialization of products for the treatment of Duchenne muscular dystrophy and myotonic dystrophy type 1, and, in March 2023, we entered into a non-exclusive license agreement with Vertex for Vertex to utilize our gene editing technology in diabetes.

ViaCyte. We entered into a research and collaboration agreement in September 2018 with ViaCyte to pursue the discovery, development and commercialization of gene-edited allogeneic stem cell therapies for the treatment of diabetes, and in July 2021, we entered into a joint development and commercialization agreement with ViaCyte, or the ViaCyte JDCA. In connection with entering into the ViaCyte JDCA, our existing research collaboration agreement with ViaCyte expired in accordance with its terms. Under the ViaCyte JDCA, we and ViaCyte are jointly developing and will commercialize product candidates and shared products for use in the treatment of diabetes type 1, diabetes type 2 and insulin dependent/requiring diabetes, or the ViaCyte Collaboration Field, throughout the world. The ViaCyte JDCA includes, among other things, provisions relating to collaboration and program governance, clinical activities for the product candidates and shared products under the agreement and continuing research by the parties in the ViaCyte

Collaboration Field. Unless otherwise mutually agreed, research costs incurred by a party will be solely borne by such party. The program expenses, as originally set forth in the research and collaboration agreement, as applicable, incurred through the date of first commercial sale of a shared product will be allocated 60% to us and 40% to ViaCyte. Following first commercial sale of a shared product, such program expenses will be shared equally between us and ViaCyte. Shared product revenues will be shared equally by us and ViaCyte. In the third quarter of 2022, Vertex announced it had acquired ViaCyte and the rights to the ViaCyte Collaboration Field, and in March 2023, we entered into an amendment to the ViaCyte JDCA pursuant to which, among other things, we adjusted certain rights and obligations of the parties thereunder.

Bayer. We entered into an option agreement in the fourth quarter of 2019 with Bayer pursuant to which Bayer has an option to co-develop and co-commercialize two products that we advance for the diagnosis, treatment, or prevention of certain autoimmune disorders, eye disorders, or hemophilia A disorders for a specified period of time, or, under certain circumstances, exclusively license such optioned products.

Other Partnerships. We have entered into a number of additional collaborations and license agreements to support and complement our hematopoietic stem cell, immuno-oncology, regenerative medicine and *in vivo* programs and platform, including agreements with: Nkarta, Inc. to co-develop and co-commercialize two donor-derived, gene-edited CAR-NK cell product candidates and a product candidate combining NK and T cells; Capsida Biotherapeutics, Inc. to develop *in vivo* gene editing therapies delivered with engineered AAV vectors for the treatment of amyotrophic lateral sclerosis and Friedreich's ataxia; Moffitt Cancer Center and Roswell Park Comprehensive Cancer Center to advance autologous CAR T programs against new targets; MaxCyte, Inc. on *ex vivo* delivery for our hemoglobinopathy and immuno-oncology programs; CureVac AG on optimized mRNA constructs and manufacturing for certain *in vivo* programs; and KSQ Therapeutics, Inc. on intellectual property for our allogeneic immuno-oncology programs.

Impacts of Coronavirus and Market Conditions on Our Business

We have been actively monitoring the coronavirus pandemic situation and its impact globally. We believe our financial results for the three months ended March 31, 2023 and 2022 were not significantly impacted by the outbreak of the coronavirus. We believe our hybrid and remote working arrangements have had limited impact on our ability to maintain internal operations during such time periods. Further, disruption of global financial markets and a recession or market correction, including as a result of the coronavirus pandemic, the recent failure of certain banks and financial institutions in the United States and globally, the ongoing military conflict between Russia and Ukraine and the related sanctions imposed against Russia, and other global macroeconomic factors such as inflation, could reduce our ability to access capital, which could, in the future, negatively affect our business and the value of our common shares.

Financial Overview

Since our inception in October 2013, we have devoted substantially all of our resources to our research and development efforts, identifying potential product candidates, undertaking drug discovery and preclinical development activities, building and protecting our intellectual property estate, organizing and staffing our company, business planning, raising capital and providing general and administrative support for these operations. To date, we have primarily financed our operations through private placements of our preferred shares, common share issuances, convertible loans and collaboration agreements with strategic partners.

While we were in a net income position in certain previous years due to certain payments associated with our collaborations with Vertex, we have a history of recurring losses and expect to continue to incur losses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly as we continue our current research programs and development activities; seek to identify additional research programs and additional product candidates; conduct initial drug application supporting preclinical studies and initiate clinical trials for our product candidates; initiate preclinical testing and clinical trials for any other product candidates we identify and develop; seek regulatory approval for our product candidates; maintain, defend, protect and expand our intellectual property estate; further develop our gene editing platform; hire additional research, clinical and scientific personnel; incur facilities costs associated with such personnel growth; develop manufacturing infrastructure and conduct related regulatory validation activities; and incur additional costs associated with operating as a public company.

Revenue Recognition

We have not generated any revenue to date from product sales and do not expect to do so in the near future. Revenue recognized for the three months ended March 31, 2023 was $100.0 million related to our receipt of an upfront payment from Vertex in connection with entering into agreements with Vertex and ViaCyte relating to the research, development, manufacture and commercialization of therapeutic products in the diabetes field. Revenue recognized for the three months ended March 31, 2022 was not material. For additional information about our revenue recognition policy, see Note 2, "Summary of Significant Accounting Policies," in our Annual Report on Form 10-K for the year ended December 31, 2022 filed with the SEC on February 21, 2023, as well as Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

Research and Development Expenses

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

- employee-related expenses, including salaries, benefits and equity-based compensation expense;
- costs of services performed by third parties that conduct research and development and preclinical and clinical activities on our behalf;
- costs of purchasing lab supplies and non-capital equipment used in our preclinical activities and in manufacturing preclinical and clinical study materials;
- consultant fees;
- facility costs, including rent, depreciation and maintenance expenses; and
- fees and other payments related to acquiring and maintaining licenses under our third-party licensing agreements.

Our external research and development expenses support our various preclinical and clinical programs, and as such we do not break down external research and development expenses further. Our internal research and development expenses consist of payroll and benefits expenses, facilities expense, and other indirect research and development expenses incurred in support of overall research and development activities and as such are not allocated to a specific development stage or therapeutic area. Research and development costs are expensed as incurred. Nonrefundable advance payments for research and development goods or services to be received in the future are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed. At this time, we cannot reasonably estimate or know the nature, timing or estimated costs of the efforts that will be necessary to complete the development of any product candidates we may identify and develop. This is due to the numerous risks and uncertainties associated with developing such product candidates, including the uncertainty of:

- successful completion of preclinical studies and IND-enabling studies;
- successful enrollment in, and completion of, clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of the product, if and when approved, whether alone or in collaboration with others;
- acceptance of the product, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

A change in the outcome of any of these variables with respect to the development of any product candidates or the subsequent commercialization of any product candidates we may successfully develop could significantly change the costs, timing and viability associated with the development of that product candidate.

Research and development activities are central to our business model. We expect to continue to incur research and development costs consistent with research and development at companies of our size and stage of development, which may increase in the foreseeable future as our current development programs progress, new programs are added and we continue to prepare regulatory filings. These increases will likely include the costs related to the implementation and expansion of clinical trial sites and related patient enrollment, monitoring, program management and manufacturing expenses for current and future clinical trials.

General and Administrative Expenses

General and administrative expenses consist primarily of employee related expenses, including salaries, benefits and equity-based compensation, for personnel in executive, finance, accounting, business development, human resources and other general and administrative functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services.

We expect to continue to incur general and administrative expenses consistent with research and development at companies of our size and stage of development, which may increase in the future to support continued research and development activities, and potential commercialization of our product candidates. In addition, we anticipate ongoing expenses related to the reimbursements of third-party patent related expenses in connection with certain of our in-licensed intellectual property.

Collaboration Expense, Net

Collaboration expense, net, consists of operating expense related to the exa-cel program under our collaboration with Vertex. Under the A&R Vertex JDCA, we have an option to defer our portion of specified costs on the exa-cel program in excess of $110.3 million for the years ended December 31, 2023 and 2024. Vertex may only recover any such deferred amounts as an offset against future profitability of the exa-cel program, determined on an annual basis in accordance with the A&R Vertex JDCA. Any such deferred amounts are capped at a specified maximum amount per year.

Other Income (Expense), Net

Other income (expense), net consists primarily of interest income earned on investments.

Results of Operations

Comparison of three months ended March 31, 2023 and 2022 (in thousands):

	Three Months Ended March 31,		Period to Period Change
	2023	2022	
Revenue:			
Collaboration revenue	$ 100,000	$ 178	$ 99,822
Grant revenue	—	762	(762)
Total revenue	100,000	940	99,060
Operating expenses:			
Research and development	99,935	118,245	(18,310)
General and administrative	22,360	28,021	(5,661)
Collaboration expense, net	42,192	30,646	11,546
Total operating expenses	164,487	176,912	(12,425)
Loss from operations	(64,487)	(175,972)	111,485
Other income, net	12,742	363	12,379
Loss before income taxes	(51,745)	(175,609)	123,864
Benefit for income taxes	(1,320)	(3,608)	2,288
Net loss	$ (53,065)	$ (179,217)	$ 126,152

Collaboration Revenue

Collaboration revenue for the three months ended March 31, 2023 was $100.0 million due to an upfront payment from Vertex. Revenue for the three months ended March 31, 2022 was not material. Please refer to Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for further information.

Research and Development Expenses

Research and development expenses were $99.9 million for the three months ended March 31, 2023, compared to $118.2 million for the three months ended March 31, 2022. The following table summarizes our research and development expenses for the three months ended March 31, 2023 and 2022, together with the changes in those items in dollars (in thousands):

	Three Months Ended March 31,		Period to Period Change
	2023	2022	
External research and development expenses	$ 34,493	$ 45,476	$ (10,983)
Employee related expenses	22,558	21,191	1,367
Facility expenses	28,400	30,780	(2,380)
Stock-based compensation expenses	11,676	14,589	(2,913)
Other expenses	693	539	154
Sublicense and license fees	2,115	5,670	(3,555)
Total research and development expenses	$ 99,935	$ 118,245	$ (18,310)

The decrease of approximately $18.3 million was primarily attributable to the following:

- $11.0 million of decreased external research and development costs, primarily associated with a decrease in variable external research and manufacturing costs;
- $3.6 million of decreased sublicense and license fees; and

- $2.9 million of decreased stock-based compensation expenses primarily due to an overall decrease in the fair value of equity awards granted in 2022 and into 2023 and subsequent reduction in stock-based compensation expense incurred.

General and Administrative Expenses

General and administrative expenses were $22.4 million for the three months ended March 31, 2023, compared to general and administrative expenses of $28.0 million for the three months ended March 31, 2022. The decrease in general and administrative expenses of $5.6 million was primarily attributable to decreased stock-based compensation expense and decreased intellectual property costs.

Collaboration Expense, Net

Collaboration expense, net, was $42.2 million for the three months ended March 31, 2023, compared to $30.6 million for the three months ended March 31, 2022. The increase of approximately $11.5 million was primarily attributable to increased manufacturing and other pre-commercial costs with regards to the exa-cel program.

Other Income, Net

Other income was $12.7 million for the three months ended March 31, 2023, compared to $0.4 million of income for the three months ended March 31, 2022. The increase was primarily due to interest income earned on cash, cash equivalents and marketable securities for the three months ended March 31, 2023.

Liquidity and Capital Resources

We have predominantly incurred losses and cumulative negative cash flows from operations since our inception. As of March 31, 2023, we had $1,889.5 million in cash, cash equivalents and marketable securities, of which approximately $2.5 million was held outside of the United States, and an accumulated deficit of $899.2 million. We anticipate that we will continue to incur losses for at least the next several years. We expect to continue to incur research and development costs and general and administrative expenses consistent with costs associated with research and development at companies of our size and stage of development, and, as a result, we will need additional capital to fund our operations, which we may raise through public or private equity or debt financings, strategic collaborations, or other sources.

In August 2019, we entered into the August 2019 Sales Agreement with Jefferies and filed our current prospectus supplement for $419.8 million in July 2021. As of March 31, 2023, we have issued and sold an aggregate of 1.1 million common shares under the current prospectus supplement at an average price of $168.79 per share for aggregate proceeds of $178.8 million, which were net of equity issuance costs of $2.4 million.

Funding Requirements

Our primary uses of capital are, and we expect will continue to be, research and development activities, manufacturing activities, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses, patent prosecution, filing, defense and intellectual property maintenance costs, and general overhead costs, including costs associated with operating as a public company. We expect to continue to incur operating expenses consistent with costs associated with research and development at companies of our size and stage of development, which may increase in the future to support continued research and development activities and potential commercialization of our product candidates.

Because most of our programs are still in early stages of development and the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete the development, manufacture and commercialization of any current or future product candidates, if approved, or whether, or when, we may achieve profitability. Until such time as we can generate substantial product revenues, if ever, we expect to finance our cash needs through a combination of equity financings, debt financings and payments received in connection with our collaboration agreements. We intend to consider opportunities to raise additional funds through the sale of equity or debt securities when market conditions are favorable to us to do so. However, the trading prices for our common shares and other biopharmaceutical companies have been highly volatile. As a result, we may face difficulties raising capital through sales of our common shares or such sales may be on unfavorable terms. In addition, a recession, depression or other sustained adverse market event, including resulting from the continued spread of the coronavirus or the recent failure of certain banks and financial institutions in the United States and globally, could materially and adversely affect our business and the value of our common shares. To the extent that we raise additional capital through the future sale of equity or debt securities, the ownership interests of our shareholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing shareholders. If we raise additional funds through collaboration arrangements in the future, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. 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.

Outlook

Based on our research and development plans and our timing expectations related to the progress of our programs, we expect our existing cash will enable us to fund our operating expenses and capital expenditures for at least the next 24 months without giving effect to any additional proceeds we may receive under our collaboration with Vertex and any other capital raising transactions we may complete. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we expect. Given our need for additional financing to support the long-term clinical development of our programs, we intend to consider additional financing opportunities when market terms are favorable to us.

Our ability to generate revenue and achieve profitability depends significantly on our success in many areas, including: developing our delivery technologies and our gene editing technology platform; selecting appropriate product candidates to develop; completing research and preclinical and clinical development of selected product candidates; obtaining regulatory approvals and marketing authorizations for product candidates for which we complete clinical trials; developing a sustainable and scalable manufacturing process for product candidates; launching and commercializing product candidates for which we obtain regulatory approvals and marketing authorizations, either directly or with a collaborator or distributor; obtaining market acceptance of our product candidates, if approved; addressing any competing technological and market developments; negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter; maintaining good relationships with our collaborators and licensors; maintaining, defending, protecting and expanding our estate of intellectual property rights, including patents, trade secrets and know-how; and attracting, hiring and retaining qualified personnel.

Cash Flows

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

	Three Months Ended March 31,		Period to Period Change
	2023	2022	
Net cash provided by (used in) operating activities	$ 8,799	$ (135,239)	$ 144,038
Net cash provided by (used in) investing activities	117,829	(114,515)	232,344
Net cash provided by financing activities	5,404	10,649	(5,245)
Effect of exchange rate changes on cash	32	(27)	59
Net increase (decrease) in cash	$ 132,064	$ (239,132)	$ 371,196

Operating Activities

Net cash provided by operating activities was $8.8 million for the three months ended March 31, 2023, compared to cash used in operating activities of $135.2 million for the three months ended March 31, 2022. The increase from a cash used in operating activities position to a cash provided by operating activities position of $144.0 million was primarily driven by a reduction in our net loss position of $126.2 million, from a net loss of $179.2 million for the three months ended March 31, 2022 to net loss of $53.1 million for the three months ended March 31, 2023 driven by revenue recognized in the connection with an upfront payment from Vertex in the first quarter of 2023. Additionally, non-cash expense decreased by $12.9 million, primarily related to a decrease in stock-based compensation expense and amortization of premiums and discounts on marketable securities, while net changes of operating assets and liabilities increased $30.8 million.

Investing Activities

Net cash provided by investing activities for the three months ended March 31, 2023 was $117.8 million, compared to net cash used in investing activities of $114.5 million for the three months ended March 31, 2022. The change from a net cash used in investing activities to a net cash provided by investing activities consisted primarily of an increase in marketable securities maturities, in addition to a reduction of purchases of marketable securities and property and equipment.

Financing Activities

Net cash provided by financing activities for the three months ended March 31, 2023 was $5.4 million, compared with $10.6 million for the three months ended March 31, 2022. Net cash provided by financing activities for the three months ended March 31, 2023 and 2022 consisted of option exercise proceeds, net of issuance costs.

Critical Accounting Policies and Significant Judgments and Estimates

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. GAAP. We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We believe that our most critical accounting policies are those relating to revenue recognition and equity-based compensation, and there have been no changes to our accounting policies discussed in our Annual Report on Form 10-K for the year ended December 31, 2022 filed with the SEC on February 21, 2023.

Recent Accounting Pronouncements

Refer to Note 1 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for a discussion of recent accounting pronouncements.

Item 3. Qualitative and Quantitative Disclosures about Market Risk

Interest Rate Sensitivity

We are exposed to market risk related to changes in interest rates. As of March 31, 2023, we had cash, cash equivalents and marketable securities of $1,889.5 million, primarily invested in U.S. treasury securities and government agency securities, corporate bonds, commercial paper and money market accounts invested in U.S. government agency securities. Due to the conservative nature of these instruments, we do not believe that we have a material exposure to interest rate risk. If interest rates were to increase or decrease by 1%, the fair value of our investment portfolio would increase or decrease by an immaterial amount.

Foreign Currency Exchange Rate Risk

As a result of our foreign operations, we face exposure to movements in foreign currency exchange rates, primarily the Swiss Franc and British Pound, against the U.S. dollar. The current exposures arise primarily from cash, accounts payable and intercompany receivables and payables. Changes in foreign exchange rates affect our consolidated statement of operations and distort comparisons between periods. To date, foreign currency transaction gains and losses have not been material to our financial statements, and we have not engaged in any foreign currency hedging transactions.

Inflation

Inflation generally affects us by increasing our cost of labor, clinical trial and manufacturing costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the three months ended March 31, 2023 and 2022.

Item 4. Controls and Procedures.

Management's Evaluation of our Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities and Exchange Act of 1934 is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of March 31, 2023, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities and Exchange Act of 1934). 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 management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of March 31, 2023, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

During the quarter ended March 31, 2023, there were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, 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.

In the ordinary course of business, we are from time to time involved in lawsuits, investigations, proceedings and threats of litigation related to, among other things, our intellectual property estate (including certain in-licensed intellectual property), commercial arrangements and other matters. Such proceedings may include quasi-litigation, *inter partes* administrative proceedings in the U.S. Patent and Trademark Office and the European Patent Office involving our intellectual property estate including certain in-licensed intellectual property. There are currently no claims or actions pending against us that, in the opinion of our management, are likely to have a material adverse effect on our business.

There have been no material developments with respect to the legal proceedings previously disclosed in "Item 3. Legal Proceedings" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022 filed with the SEC on February 21, 2023.

Item 1A. Risk Factors.

We are updating and supplementing our risk factors previously disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2022 filed with the SEC on February 21, 2023 to add the following new risk factor:

Conditions in the banking system and financial markets, including the failure of banks and financial institutions, could have an adverse effect on our operations and financial results.

Actual events involving limited liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10 and March 12, 2023, the Federal Deposit Insurance Corporation took control and was appointed receiver of Silicon Valley Bank, or SVB, and Signature Bank and Silvergate Capital Corp., or Silvergate Capital, respectively, after each bank was unable to continue their operations. Since then, additional financial institutions have experienced similar failures and have been placed into receivership. It is possible that other banks will face similar difficulty in the future. These events exposed vulnerabilities in the banking sector, including legal uncertainties, significant volatility and contagion risk, and caused market prices of regional bank stocks to plummet.

As of the date of this Quarterly Report on Form 10-Q, our exposure to SVB, Signature Bank, Silvergate Capital, and other banks in financial difficulty is immaterial; however, we are unable to predict the extent or nature of the impacts of these evolving circumstances at this time. If, for example, other banks and financial institutions enter receivership or become insolvent in the future in response to financial conditions affecting the banking system and financial markets, our ability to access our existing cash, cash equivalents and investments may be threatened. While it is not possible at this time to predict the extent of the impact that high market volatility and instability of the banking sector could have on economic activity and our business in particular, the failure of other banks and financial institutions and the measures taken by governments, businesses and other organizations in response to these events could adversely impact our business, financial condition and results of operations.

There have been no other material changes to the risk factors previously disclosed in Part I, Item 1A (Risk Factors) of our Annual Report on Form 10-K for the year ended December 31, 2022. Please refer to the complete Part I, Item 1A of our Annual Report for additional risks and uncertainties we are facing that may have a material adverse effect on our business prospects, financial condition and results of operations. In addition to the risks described in our Annual Report on Form 10-K, you should carefully consider the other information set forth in this Form 10-Q and the information in our other filings with the SEC, as they could materially affect our business, financial condition or future results of operations.

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

None.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth in the Exhibit Index below.

Exhibit Number	Description of Document
10.1	Employment Agreement, dated March 14, 2023, by and between CRISPR Therapeutics, Inc., and Raju Prasad, Ph.D. (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on March 14, 2023).
10.2†^	Non-Exclusive License Agreement, dated March 23, 2023, by and between Vertex Pharmaceuticals Incorporated and CRISPR Therapeutics AG (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on March 27, 2023).
31.1*	Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1*+	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the inline XBRL document.
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104*	Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101)

* Filed herewith.

† Certain portions of this exhibit have been omitted because they are not material and the registrant customarily and actually treats that information as private or confidential.

^ Certain exhibits and schedules to these agreements have been omitted pursuant to Item 601 of Regulation S-K. The registrant will furnish copies of any of the exhibits and schedules to the Securities and Exchange Commission upon request.

\+ The certification attached as Exhibit 32.1 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of CRISPR Therapeutics AG 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.

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.

CRISPR Therapeutics AG

Dated: May 8, 2023

By: /s/ Samarth Kulkarni
Samarth Kulkarni
Chief Executive Officer
(Principal Executive Officer)

Dated: May 8, 2023

By: /s/ Raju Prasad
Raju Prasad
Chief Financial Officer
(Principal Financial Officer)

Exhibit 31.1

CERTIFICATIONS

I, Samarth Kulkarni, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CRISPR Therapeutics AG;

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: May 8, 2023

By: /s/ Samarth Kulkarni
Samarth Kulkarni
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Raju Prasad, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of CRISPR Therapeutics AG;

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: May 8, 2023

By: /s/ Raju Prasad
Raju Prasad
Chief Financial Officer
(Principal Financial and 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 CRISPR Therapeutics AG (the "Company") for the period ended March 31, 2023 as filed with the Securities and Exchange Commission on or about the date hereof (the "Report"), the undersigned officers of the Company hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to his or her 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 as of, and for, the periods presented in the Report.

/s/ Samarth Kulkarni

Samarth Kulkarni
Chief Executive Officer
(Principal Executive Officer)

May 8, 2023

/s/ Raju Prasad

Raju Prasad
Chief Financial Officer
(Principal Financial and Accounting Officer)

May 8, 2023