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, 2020

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	Name of each exchange on which registered
Common Shares, CHF 0.03 par value	**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 April 24, 2020, there were 61,017,707 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" is a registered trademark of CRISPR Therapeutics AG. The trademarks for "CTX001 ™," "CTX110 ™," "CTX120 ™," and "CTX130 ™" are pending in the United States and the trademark for "CRISPR Therapeutics" is pending in the European Union, Switzerland and the United Kingdom. Other brands, logos, names and 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, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights to these trademarks, service marks and trade names.

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:

- *potential impacts due to the coronavirus pandemic such as delays, interruptions or other adverse effects to clinical trials, delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy, and the overall impact of the coronavirus pandemic on our business, financial condition and results of operations;*
- *the safety, efficacy and clinical progress of our various clinical programs including those for CTX001 ™, CTX110 ™, CTX120 ™ and CTX130 ™;*
- *the status of clinical trials, development timelines 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 for CTX001, CTX110, CTX120 and CTX130, and our research and development programs, including delays or disruptions in clinical trials, non-clinical experiments and investigational new drug application-enabling studies;*
- *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 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 12, 2020, 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, 2020	**December 31, 2019**
Assets		
Current assets:		
Cash and cash equivalents	$ 889,712	$ 943,771
Accounts receivable	25,117	99
Prepaid expenses and other current assets	14,453	43,677
Total current assets	929,282	987,547
Property and equipment, net	31,773	31,330
Intangible assets, net	221	235
Restricted cash	5,041	5,041
Operating lease assets	40,321	41,502
Other non-current assets	662	1,097
Total assets	$ 1,007,300	$ 1,066,752
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 11,277	$ 5,944
Accrued expenses	21,924	30,180
Deferred revenue, current	803	960
Accrued tax liabilities	284	583
Operating lease liabilities	9,005	8,489
Other current liabilities	11,793	10,950
Total current liabilities	55,086	57,106
Deferred revenue, non-current	11,776	11,776
Operating lease liabilities, net of current portion	42,390	44,050
Other non-current liabilities	11,951	14,395
Total liabilities	121,203	127,327
Commitments and contingencies, see Note 4		
Shareholders' equity:		
Common shares, CHF 0.03 par value, 103,901,006 and 103,901,006 shares authorized at March 31, 2020 and December 31, 2019, respectively, 61,122,431 and 61,034,025 shares issued at March 31, 2020 and December 31, 2019, respectively 60,890,035 and 60,783,799 shares outstanding at March 31, 2020 and December 31, 2019, respectively.	1,850	1,847
Treasury shares, at cost, 232,396 and 250,226 shares at March 31, 2020 and December 31, 2019, respectively.	(63)	(63)
Additional paid-in capital	1,178,770	1,162,345
Accumulated deficit	(294,442)	(224,711)
Accumulated other comprehensive (loss) income	(18)	7
Total shareholders' equity	886,097	939,425
Total liabilities and shareholders' equity	$ 1,007,300	$ 1,066,752

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,	
	2020	2019
Collaboration revenue (1)	$ 157	$ 328
Operating expenses:		
Research and development (2)	54,193	33,822
General and administrative	19,550	14,929
Total operating expenses	73,743	48,751
Loss from operations	(73,586)	(48,423)
Other income (expense):		
Loss from equity method investment	-	(1,025)
Other income, net	4,232	1,125
Total other income, net	4,232	100
Net loss before income taxes	(69,354)	(48,323)
Provision for income taxes	(377)	(85)
Net loss	(69,731)	(48,408)
Foreign currency translation adjustment	(25)	8
Comprehensive loss	$ (69,756)	$ (48,400)
Net loss per share attributable to common shareholders—basic and diluted	$ (1.15)	$ (0.93)
Weighted-average common shares outstanding used in net loss per share attributable to common shareholders—basic and diluted	60,847,683	52,093,208
(1) Including the following revenue from a related party, see Notes 5 & 10:	$ —	$ 328
(2) Including the following research and development expense with a related party, see Notes 5 & 10:	$ —	$ 7,587

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

CRISPR Therapeutics AG
Consolidated Statements of Shareholders' Equity
(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 Income (Loss)	Total Shareholders' Equity
Balance at December 31, 2018	51,852,862	1,584	307,936	(57)	682,245	(291,569)	(8)	392,195
Issuance of common shares, net of issuance costs of $1.2 million	631,580	—	—	—	23,472	—	—	23,472
Vesting of restricted shares	9,288	—	—	—	15	—	—	15
Exercise of vested options	141,915	5	—	—	1,827	—	—	1,832
Stock-based compensation expense	—	—	—	—	10,696	—	—	10,696
Other comprehensive income	—	—	—	—	—	—	8	8
Net loss	—	—	—	—	—	(48,408)	—	(48,408)
Balance at March 31, 2019	52,635,645	$ 1,589	307,936	$ (57)	$ 718,255	$ (339,977)	$ -	$ 379,810
Balance at December 31, 2019	60,783,799	1,847	250,226	(63)	1,162,345	(224,711)	7	939,425
Vesting of restricted shares	5,000	—	—	—	—	—	—	-
Exercise of vested options	83,406	3	—	—	1,385	—	—	1,388
Stock-based compensation expense	—	—	—	—	14,151	—	—	14,151
Issuance of common shares related to license agreement	17,830	—	(17,830)	—	889	—	—	889
Other comprehensive loss	—	—	—	—	—	—	(25)	(25)
Net loss	—	—	—	—	—	(69,731)	—	(69,731)
Balance at March 31, 2020	60,890,035	$ 1,850	232,396	$ (63)	$ 1,178,770	$ (294,442)	$ (18)	$ 886,097

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,	
	2020	**2019**
Operating activities:		
Net loss	$ (69,731)	$ (48,408)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation and amortization	2,091	812
Equity-based compensation	14,151	9,671
Loss from equity method investment	-	1,025
Other expense, non-cash	890	-
Changes in:		
Accounts receivable	(25,018)	(243)
Prepaid expenses and other assets	29,659	(3,051)
Accounts payable and accrued expenses	(2,496)	(3,324)
Deferred revenue	(157)	(25)
Operating lease assets and liabilities	37	—
Other liabilities, net	(1,601)	(194)
Net cash used in operating activities	(52,175)	(43,737)
Investing activities:		
Purchase of property and equipment	(2,991)	(1,097)
Net cash used in investing activities	(2,991)	(1,097)
Financing activities:		
Proceeds from issuance of common shares, net of issuance costs	-	23,894
Proceeds from exercise of options, net of issuance costs	1,132	1,832
Net cash provided by financing activities	1,132	25,726
Effect of exchange rate changes on cash	(25)	8
Decrease in cash	(54,059)	(19,100)
Cash, cash equivalents and restricted cash, beginning of period	948,812	459,812
Cash, cash equivalents and restricted cash, end of period	$ 894,753	$ 440,712
Supplemental disclosure of non-cash investing and financing activities		
Property and equipment purchases in accounts payable and accrued expenses	$ 1,340	$ 579
Equity issuance costs in accounts payable and accrued expenses	$ 39	$ 181

	As of March 31,	
Reconciliation to amounts within the condensed consolidated balance sheets	**2020**	**2019**
Cash and cash equivalents	$ 889,712	$ 437,549
Restricted cash	5,041	3,163
Cash, cash equivalents and restricted cash at end of period	894,753	440,712

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 GAAP.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. Prior to December 13, 2019, the Company accounted for its 50% investment in Casebia Therapeutics Limited Liability Partnership, or Casebia, under the equity method. As described in Note 5, on December 13, 2019, Casebia became a fully-owned subsidiary and, as a result, the Company consolidated Casebia's financial results from that date forward. 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, 2020 and 2019.

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, 2019, which are contained in the 2019 Annual Report on Form 10-K filed with the Securities and Exchange Commission, or the SEC, on February 12, 2020.

Use of Estimates

The preparation of financial statements in conformity with 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, 2020 are consistent with those discussed in Note 2 to the consolidated financial statements in the Company's 2019 Annual Report on Form 10-K filed with the SEC on February 12, 2020, except with respect to the Company's policy on credit losses noted within the "Recently adopted accounting standards" section below.

Recently Adopted Accounting Standards

Credit Losses

On January 1, 2020, the Company adopted ASU 2016-13, *Financial Instruments – Credit Losses (Topic 326): Measurement of Credit Losses on Financial Statements* , or ASC 326. The new standard requires that expected credit losses relating to financial assets measured on an amortized cost basis and available-for-sale debt securities be recorded through an allowance for credit losses. It also limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and also requires the reversal of previously recognized credit losses if fair value increases. The targeted transition relief standard allows filers an option to irrevocably elect the fair value option of ASC 825-10 , *Financial Instruments-Overall* , applied on an instrument-by-instrument basis for eligible instruments. The Company adopted ASC 326 using the modified retrospective method for all financial assets measured at amortized cost. The adoption of ASU 2016-13 did not have a material impact on the Company's financial position or results of operations upon adoption.

2. Property and Equipment, net

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

	As of	
	March 31, 2020	December 31, 2019
Computer equipment	$ 727	$ 727
Furniture, fixtures and other	3,215	3,215
Laboratory equipment	18,555	16,640
Leasehold improvements	21,400	21,400
Construction work in process	1,999	1,394
Total property and equipment, gross	45,896	43,376
Accumulated depreciation	(14,123)	(12,046)
Total property and equipment, net	$ 31,773	$ 31,330

Depreciation expense for the three months ended March 31, 2020 and 2019 was $2.1 million and $1.0 million, respectively.

3. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	As of	
	March 31, 2020	December 31, 2019
Payroll and employee-related costs	$ 5,431	$ 15,229
Research costs	11,113	9,434
Licensing fees	—	750
Professional fees	1,694	2,040
Intellectual property costs	2,780	2,311
Accrued property and equipment	148	407
Other	758	9
Total	$ 21,924	$ 30,180

4. Commitments and Contingencies

Future Lease Commitments

The Company has entered into certain leasing commitments, for which, right of use assets and right of use liabilities are not reflected on the consolidated balance sheet as the leases have not yet commenced.

In November 2019, the Company, together with one of its partners, committed to making payments to a clinical manufacturing organization under a lease arrangement. The lease arrangement is expected to commence in the second half of 2020, at which time an upfront payment of $2.6 million is due. In addition, the Company and its partner have committed to paying approximately $3.7 million in annual rental payments for a five-year period following commencement. All payments will be split equally between the Company and its partner.

Litigation

In the ordinary course of business, the Company is from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements, employment and other matters. While the outcome of those proceedings and claims cannot be predicted with certainty, the Company is not party to any legal or arbitration proceedings that may have significant effects on its financial position. It is not a party to any material proceedings in which any director, member of executive management or affiliate of the Company is either a party adverse to it or its subsidiaries or has a material interest adverse to it or its subsidiaries.

Letters of Credit

As of March 31, 2020, the Company had restricted cash of $5.0 million representing letters of credit securing the Company's obligations under certain leased facilities in Cambridge, Massachusetts, as well as certain credit card arrangements. The letters of credit are secured by cash held in a restricted depository account. The cash deposit is recorded in restricted cash in the accompanying consolidated balance sheet as of March 31, 2020.

Research, 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 research 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 Pharmaceuticals Incorporated and certain of its subsidiaries, or Vertex, is eligible to receive up to $395.0 million in potential specified research, development, regulatory and commercial milestones and tiered single-digit royalties on future net sales. Refer to Note 5 for further discussion on the Company's arrangements with Vertex.

5. Significant Contracts

Agreements with Vertex Pharmaceuticals Incorporated and certain of its subsidiaries

Summary

On October 26, 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.

On December 12, 2017, the Company and Vertex entered into Amendment No. 1 to the 2015 Collaboration Agreement, or Amendment No. 1, and the Joint Development Agreement, or the JDA. Amendment No. 1, among other things, modified certain definitions and provisions of the 2015 Collaboration Agreement to make them consistent with the JDA and clarified how many options are exercised (or deemed exercised) in connection with certain targets specified under the 2015 Collaboration Agreement. Amendment No. 1 also amended other provisions of the 2015 Collaboration Agreement, including the expiration terms.

In connection with the 2015 Collaboration Agreement, Vertex made a nonrefundable upfront payment of $75.0 million. Under the 2015 Collaboration Agreement, Vertex agreed to fund the discovery activities conducted pursuant to the agreement while retaining options to co-exclusive and exclusive licenses. In December 2017, upon execution of the JDA and Amendment No. 1, Vertex exercised its option to obtain a co-exclusive license to develop and commercialize hemoglobinopathy and beta-globin targets. As such, for potential hemoglobinopathy treatments, including treatments for sickle cell disease, the Company and Vertex will share equally all research and development costs and worldwide revenues. In connection with the JDA, the Company received a $7.0 million up-front payment from Vertex and subsequently received a one-time low seven-digit milestone payment upon the dosing of the second patient in a clinical trial with the initial product candidate. In addition, upon execution of the JDA and Amendment No. 1, it was clarified that Vertex may elect to license up to four remaining targets, for which it will lead global development and commercialization activities and the Company received the right to receive up to $420.0 million in development, regulatory and commercial milestones and royalties on net product sales for each of the targets (inclusive of $10 million due upon exercise of each exclusive option).

In June 2019, the Company and Vertex entered into a series of agreements, which closed on July 23, 2019, 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. Under the terms of the 2019 Collaboration Agreement, the Company received an upfront, nonrefundable payment of $175.0 million. In addition, the Company is eligible to receive potential aggregate payments of up to $825.0 million based upon the successful achievement of specified research, 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. For the DMD program, Vertex is responsible for all research, development, manufacturing and commercialization activities and all related costs. For the DM1 program, the Company will perform specified guide RNA research and Vertex is responsible for all other research, development, manufacturing and commercialization costs. Upon Investigational New Drug, or IND, application 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.

In connection with the execution of the 2019 Collaboration Agreement, the Company and Vertex entered into a second amendment to the 2015 Collaboration Agreement, or Amendment No. 2. Among other things, Amendment No. 2 modified certain definitions and provisions of the 2015 Collaboration Agreement to make them consistent with the 2019 Collaboration Agreement and set forth the number and identity of the collaboration targets under the 2015 Collaboration Agreement. The Company and Vertex agreed that one of the four remaining options under the 2015 Collaboration Agreement, as amended, would not be exercised; instead, the Company will reacquire the exclusive rights and will conduct research and development activities for the specified target. Vertex will have the option to co-develop and co-commercialize the specified target upon IND filing in exchange for payment of 50% of research and development costs incurred by the Company from the effective date of the agreement through IND filing. If Vertex does not exercise its option to co-develop and co-commercialize the specified target, Vertex is eligible to receive up to $395.0 million in potential specified research, development, regulatory and commercial milestones and tiered single-digit royalties on future net sales.

In October 2019, Vertex exercised the remaining three options granted to it under the 2015 Collaboration Agreement to exclusively license the collaboration targets developed under the 2015 Collaboration Agreement, resulting in a payment of $30.0 million to the Company in the fourth quarter of 2019. The Company achieved the first milestone under the 2019 Collaboration Agreement in the first quarter of 2020 and in connection therewith received a payment of $25.0 million in April 2020.

Accounting for the Vertex Agreements

The 2015 Collaboration Agreement, Amendment No. 1, and JDA are collectively the “2015 Agreements” and the 2019 Collaboration Agreement and Amendment No. 2. are collectively the “2019 Agreements.” The 2015 Collaboration Agreement, Amendment No. 1, Amendment No. 2, JDA and 2019 Collaboration Agreement are collectively the “Vertex Agreements.”

The Vertex 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.

Accounting Analysis Under ASC 606

Accounting for the 2019 Agreements

Identification of the Contract

The 2019 Agreements represented a contract modification to the 2015 Agreements. As a result, the 2019 Agreements and the 2015 Agreements are combined for accounting purposes and treated as a single arrangement.

Identification of Performance Obligations

The Company concluded the following material promises were both capable of being distinct and distinct within the context of the Vertex Agreements and represented separate performance obligations: (i) an exclusive license for worldwide rights for DMD gene editing products, or DMD License; (ii) an exclusive license for worldwide rights for DM1 gene editing products, or DM1 License; (iii) the performance of specified guide RNA research for DM1, or DM1 R&D Services; (iv) a material right representing the option to obtain a co-exclusive development and commercialization license for a specified target, or Specified Target Option; (v) three material rights representing the option for up to three exclusive licenses to develop and commercialize the collaboration targets, or Collaboration Target Options; and (vi) the waiving of Vertex’s material right associated with its option to a fourth exclusive license in connection with the Company’s reacquisition of exclusive rights to the specified target.

Determination of Transaction Price

The overall transaction price was determined based on the remaining transaction price from the 2015 Agreements, as well as the transaction price from the 2019 Agreements. The transaction price includes variable consideration estimated using the most likely amount methodology. As such, the Company determined the transaction price totaling $268.6 million was comprised of: (i) $57.8 million of pre-existing deferred revenue from the 2015 Agreements; (ii) non-cash consideration of $10.0 million related to the waiving of Vertex's material right associated with its option to a fourth exclusive license in connection with the Company's reacquisition of exclusive rights to the specified target; (iii) an upfront payment of $175.0 million; (iv) variable consideration of $25.0 million which represented the Company's estimate related to a near-term research and development milestone for which the Company determined that it is not probable that a significant reversal of cumulative consideration will occur at the onset of the transaction; and (v) variable consideration of $0.8 million which represents the Company's estimate of payments from Vertex for DM1 R&D Services.

The Company determined that all other possible variable consideration resulting from milestones and royalties discussed above was fully constrained as of March 31, 2020. The Company will re-evaluate the transaction price in each reporting period.

Allocation of Transaction Price to Performance Obligations

The selling price of each performance obligation was determined based on the Company's estimated standalone selling price, or the ESSP. The Company developed the ESSP for all the performance obligations included in the Vertex Agreements with the objective of determining the price at which it would sell such an item if it were to be sold regularly on a standalone basis. The Company then allocated the transaction price to each performance obligation on a relative standalone selling price basis.

The ESSP for the DMD License and DM1 License was determined to be $224.6 million and $76.2 million, respectively. The ESSP was determined based on probability and present value adjusted cash flows from projected worldwide net profit for each of the respective programs based on probability assessments, projections based on internal forecasts, industry data, and information from other guideline companies within the same industry and other relevant factors. On a relative basis, $151.1 million and $51.3 million of the transaction price was allocated to the DMD License and DM1 License, respectively.

The ESSP for the Specified Target Option material right was determined to be $17.5 million, which was based on the incremental discount between (i) the value of the probability and present value adjusted cash flows from the equal sharing of projected worldwide net profit increased by the value of the option provided to Vertex less (ii) the expected exercise price at the time of option exercise. The present value adjusted cash flows also considered projections based on internal forecasts, industry data, and information from other guideline companies within the same industry and other relevant factors. On a relative basis, $11.8 million of the transaction price was allocated to the Specified Target Option material right.

The ESSP for each of the three Collaboration Target Option material rights was determined to be $25.0 million, $22.2 million and $22.2 million, respectively, which was determined based on the probability and present value adjusted cash flows from milestone payments owed for exclusive licenses, less the price paid to exercise each option. On a relative basis, $46.7 million of the transaction price was allocated to the Collaboration Target Option material rights.

The aforementioned ESSPs reflect the level of risk and expected probability of success inherent in the nature of the associated research area.

The ESSP for the waiving of Vertex's material right associated with its option to a fourth exclusive license under the 2015 Agreements was determined to be $10.0 million, or the contractual value of the option. On a relative basis, $6.7 million of the transaction price was allocated to the waiving of Vertex's material right associated with its option to a fourth exclusive license under the 2015 Agreements.

The ESSP for the DM1 R&D Services was determined to be $1.7 million, which was based on estimates of the associated effort and cost of the services, adjusted for a reasonable profit margin that would be expected to be realized under similar contracts. On a relative basis, $1.1 million of the transaction price was allocated to the DM1 R&D Services.

Recognition of Revenue

The Company determined that the DMD License and DM1 License represent 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. As such, the revenue related to the licenses was recognized at the point in time in which they were delivered during the third quarter of 2019.

The revenue allocated to the waiving of Vertex's material right associated with its option to a fourth exclusive license in connection with Company's reacquisition of exclusive rights to the specified target was recognized at the point in time in which the option was waived, on the effective date of the 2019 Agreements.

The Company concluded that the Specified Target Option and Collaboration Target Options were considered material rights under the Vertex Agreements. Revenue related to the three Collaboration Target Options material right was recognized at the point in time in which Vertex exercised the Collaboration Target Options, which occurred in the fourth quarter of 2019. Revenue related to the Specified Target Option will be recognized at the point in time in which the option is exercised.

The Company recognizes revenue related to the DM1 R&D Services over time as the services are rendered, which is expected to be over an 18-month period from the effective date of the 2019 Agreements.

Accounting for the 2015 Agreements (prior to the execution of the 2019 Agreements)

On January 1, 2018, the Company adopted ASC 606 using the modified retrospective approach. The Company applied the practical expedient in ASC 606-10-65-1 in identifying the satisfied and unsatisfied performance obligations, determining the transaction price and allocating the transaction price under the practical expedient in ASC 606 . There was no significant impact on revenue recognized under ASC 606 and the prior revenue recognition as a result of the adoption.

Identification of the Contract

Amendment No. 1 and the JDA represented a contract modification to the 2015 Collaboration Agreement. As a result, the 2015 Agreements are combined for accounting purposes and treated as a single arrangement.

Identification of Performance Obligations

The Company concluded the following material promises were both capable of being distinct and distinct within the context of the 2015 Agreements and represented separate performance obligations: (i) the non-exclusive research license; (ii) four material rights representing the option for up to four exclusive licenses to develop and commercialize the collaboration targets; (iii) a combined performance obligation representing the co-exclusive research license, and a development and commercialization license to develop and commercialize hemoglobinopathies and beta-globin targets; and (iv) the performance of R&D Services.

Determination of Transaction Price

The overall transaction price was comprised of: (i) original upfront payment of $75.0 million, (ii) an upfront payment of $7.0 million under the JDA, and (iii) $19.3 million of variable consideration associated with the R&D services.

The Company determined that all other possible variable consideration resulting from milestones and royalties discussed above was fully constrained at the time of the transaction.

Allocation of Transaction Price to Performance Obligations

The selling price of each performance obligation was determined based on the Company's ESSP. The Company developed the ESSP for all the performance obligations included in the 2015 Agreements with the objective of determining the price at which it would sell such an item if it were to be sold regularly on a standalone basis. The Company then allocated the transaction price to each performance obligation on a relative standalone selling price basis.

The ESSP for R&D Services was determined to be $19.3 million. The Company developed the ESSP for the R&D Services primarily based on the nature of the services to be performed and estimates of the associated effort and cost of the services, adjusted for a reasonable profit margin that would be expected to be realized under similar contracts. The Company allocated $19.3 million of the transaction price to R&D Services.

The Company's ESSP for each of the remaining material rights to obtain an exclusive license to develop and commercialize a single collaboration target are $45.6 million, $38.4 million, $17.3 million and $17.3 million for a total of $118.6 million. ESSPs for these items were determined based on the probability and present value adjusted cash flows from milestone payments owed for exclusive licenses, less the price paid to exercise each option. On a relative basis, $57.7 million of the transaction price was allocated to these material rights.

The Company's ESSP for the co-exclusive research license and the development and commercialization licenses for hemoglobinopathy and beta-globin targets is $48.9 million. The ESSP for this item was determined based on probability and present value adjusted cash flows from the equal sharing of projected worldwide net profit. ESSP reflects the level of risk and expected probability of success inherent in the nature of the associated research area. On a relative basis, $23.8 million of the transaction price was allocated to the co-exclusive research license and the development and commercialization licenses for hemoglobinopathy and beta-globin targets.

The Company used a market-based approach to determine the ESSP of the non-exclusive research license of $1.0 million. The Company determined ESSP by use of comparative data, including in-licensed research agreements negotiated and executed within the Company. On a relative basis, $0.5 million of the transaction price was allocated to the non-exclusive research license.

The aforementioned ESSPs reflect the level of risk and expected probability of success inherent in the nature of the associated research area.

Recognition of Revenue

The Company determined that the non-exclusive research license is symbolic intellectual property as Vertex receives value from the license through the Company's ongoing activities, and, as such, the revenue related to the non-exclusive research license was recognized ratably over the term of the arrangement. Upon the execution of the JDA, a co-exclusive research, development and commercialization license was granted for hemoglobinopathy and beta-globin targets. The Company determined that the revenue related to these licenses was recognized at a point in time, in which they were delivered at inception of the JDA in December 2017. As Vertex has the material right in its option to obtain four additional exclusive licenses to develop and commercialize four additional collaboration targets, the Company determined that consideration allocated to these material rights would be included in the transaction price of the exclusive license and recognized at a point in time, upon the exercise of the option by Vertex or expiration. As the Company has a right to consideration from Vertex in an amount that corresponds directly with the value of the Company's performance completed to date for the R&D services, the Company recognized revenue related to the R&D services as invoiced, in line with the practical expedient in ASC 606-10-55-18.

Revenue recognized in connection with the Vertex Agreements

Revenue recognized under the 2019 Agreements and revenue recognized under the 2015 Agreements for the three months ended March 31, 2020 and 2019, respectively, was not material.

As of March 31, 2020, there was $0.8 million of current deferred revenue related to the collaboration with Vertex compared to $0.9 million as of December 31, 2019. As of March 31, 2020, there was $11.8 million of non-current deferred revenue related to the collaboration with Vertex, which is unchanged from December 31, 2019. The transaction price allocated to the remaining performance obligations was $12.6 million.

Milestones under the Vertex Agreements

The Company has evaluated the milestones that may be received in connection with the Vertex Agreements. As discussed above, 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 the fourth quarter of 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 $800.0 million based upon the successful achievement of specified research, development, regulatory and commercial milestones for the DMD and DM1 programs. As discussed above, the first research milestone of $25.0 million was included in the transaction price. T his amount was recorded as a contract asset within prepaid expenses and other current assets on the condensed consolidated balance sheet at December 31, 2019. This milestone was achieved during the first quarter of 2020 and paid during the second quarter of 2020. As of March 31, 2020, the $25.0 million milestone was recorded in accounts receivable on the condensed consolidated balance sheet. 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.

Each of the remaining milestones are fully constrained as of March 31, 2020. 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

In connection with the 2019 Agreements, the Company identified the following collaborative elements, which were unchanged as those identified with the 2015 Agreements and are accounted for under ASC 808: (i) development and commercialization services for shared products; (ii) R&D Services for follow-on products; and (iii) committee participation. The related impact of the cost sharing associated with research and development is included in research and development expense. Expenses related to services performed by the Company are classified as research and development expense. Payments received from Vertex for partial reimbursement of expenses are recorded as a reduction of research and development expense.

During the three months ended March 31, 2020 and 2019, the Company recognized $9.1 million and $7.1 million of research and development expense related to the Vertex Agreements. Research and development expense for the three months ended March 31, 2020 and 2019 was net of $5.5 million and $4.5 million of reimbursements from Vertex, respectively.

Accounting Analysis under ASC 730

In connection with the 2019 Vertex Agreements, the Company and Vertex agreed that one of the four remaining options under the 2015 Agreements, as amended, would not be exercised; instead, the Company will conduct research and development activities for a specified target. Vertex will have the option to co-develop and co-commercialize the specified target upon IND filing in exchange for payment of 50% of research and development costs incurred by the Company from the effective date of the agreement through IND filing. If Vertex does not exercise its option to do so within a specified time period, Vertex is eligible to receive up to $395.0 million in potential specified research, development, regulatory and commercial milestones and tiered single-digit royalties on future net sales.

In connection therewith, the Company determined that in order for the Company to obtain the right to conduct research and development activities on the specified target, the Company had waived its right to receive an option exercise payment of $10.0 million from Vertex, which was included as non-cash consideration in the transaction price for the 2019 Agreements described above. The Company then subsequently reacquired its rights to the specified target by waiving payment owed by Vertex of $10.0 million for a license that represents in-process research and development and therefore, $10.0 million of non-cash consideration was fully expensed upon the execution of the 2019 Agreements. The Company also determined that research and development services through IND for the specified target and any payment of future development and commercialization milestones, as well as sales-based milestones and royalties for the specified target, would be accounted for as research and development costs under ASC 730 and expensed as incurred. In addition, the Company also determined that should the Company elect its option to co-develop and co-commercialize all DM1 products globally, it will record the option fee as research and development expense upon exercise.

Joint Venture with Bayer Healthcare LLC

Summary

On December 19, 2015, the Company entered into an agreement with Bayer, to establish a joint venture to focus on the research and the development of new therapeutics to cure blood disorders, blindness and congenital heart disease. On February 12, 2016, the Company and Bayer completed the formation of the joint venture entity, Casebia. Bayer and the Company each received a 50% equity interest in the entity in exchange for their respective contributions to the entity. At that time, the Company also entered into a separate service agreement with Casebia, under which the Company agreed to provide compensated research and development services. Collectively, these agreements are referred to as the “2015 Casebia Agreements.”

On December 13, 2019, the Company, Bayer and Casebia entered into a series of transactions by which, among other things, the Company acquired 100% of the partnership interests in Casebia, or the Retirement Agreement, the Company and Bayer terminated their joint venture, or the Joint Venture Termination Agreement, and the Company and Bayer entered into a new option agreement, or the 2019 Option Agreement. Collectively, these agreements are referred to as the “2019 Casebia Agreements.”

In connection with the Retirement Agreement, Casebia retired Bayer’s outstanding partnership interests in exchange for $22.0 million less certain estimated interim operating expenses of $6.0 million, and the Company acquired 100% of the partnership interests in Casebia.

In connection with entering into the Retirement Agreement, the Company, Bayer and Casebia entered into the Joint Venture Termination Agreement. In connection therewith, the Company and Bayer agreed to terminate the Joint Venture Agreement from December 2015. Under the Joint Venture Termination Agreement, Casebia-owned patents are co-owned by the Company and Bayer, subject to certain exclusive licenses granted therein. Under the Joint Venture Termination Agreement, the Company and Bayer each retained rights to their respective contributed intellectual property.

In connection with entering into the Retirement Agreement and the Joint Venture Termination Agreement, the Company and Bayer also entered into the 2019 Option Agreement, under which, among other things, the Company committed to invest a specified

amount in certain research and development activities as described under “Accounting Analysis – Accounting for 2019 Casebia Agreements”. In addition, Bayer has an option (exercisable during a specified exercise period defined by future events, but in no event longer than 5 years after the effective date of the 2019 Option Agreement) to co-develop and co-commercialize two products for the diagnosis, treatment or prevention of certain autoimmune disorders, eye disorders or hemophilia A disorders. In the event Bayer elects to co-develop and co-commercialize a product, the parties will negotiate and enter into a co-development and co-commercialization agreement, or the Co-Commercialization Agreement, for such product, and Bayer would be responsible for 50 % of the research and development costs incurred by the Company for such product going forward. Bayer would receive 50 % of all profits from sales of such product and would be responsible for 50 % of all losses.

If Bayer elects to exercise its option to co-develop and co-commercialize a product, Bayer will make a one-time $20.0 million payment, or the Option Payment, to the Company that will become non-refundable once the parties execute a Co-Commercialization Agreement with respect to such optioned product. The Option Payment is payable only once with respect to the first time Bayer exercises an option under the 2019 Option Agreement.

In addition, following Bayer’s exercise of its option and/or the execution of the Co-Commercialization Agreement for an optioned product, for a period beginning on the effective date of such Co-Commercialization Agreement and ending on the earlier of the three month anniversary of such effective date or during the 90-day negotiation process of such Co-Commercialization Agreement, Bayer has a right to negotiate an exclusive license to develop and commercialize such optioned product. If Bayer exercises such right, the parties will enter into an exclusive license agreement for such optioned product on terms mutually agreeable to the parties. Further, the Option Payment paid for such optioned product would become credited against payments due under such exclusive license or any other exclusive license entered into in connection with the 2019 Option Agreement.

Either party may terminate the 2019 Option Agreement upon the other party’s material breach, subject to specified notice and cure provisions. The Company may also terminate the 2019 Option Agreement in the event Bayer commences or participates in any action or proceeding challenging the validity or enforceability of any Company patent necessary or useful for the research, development, manufacture or commercialization of a product that is the subject of the 2019 Option Agreement. Bayer may also terminate the 2019 Option Agreement upon the Company’s bankruptcy or insolvency, or for convenience at any time, after giving written notice.

Accounting Analysis

Accounting for the 2015 Casebia Agreements

During 2016, the Company recorded an equity method investment of $36.5 million equal to the fair value of the Company’s interest in Casebia and subsequently recorded unrealized equity method losses for the same amount. The Company had no further contractual obligations to provide cash financing to Casebia and accordingly, no additional losses were recorded beyond the initial equity amount. Casebia’s net losses were $14.2 million for the three months ended March 31, 2019 and unrecognized equity method losses in excess of the Company’s equity method investment in Casebia were $51.9 million as of March 31, 2019.

The remaining performance obligations prior to the 2019 Casebia Agreements included research and development services, which were recorded as revenue under ASC 606, and cost sharing activities with Casebia related to shared research and technology licenses, which were recorded as a cost/profit sharing arrangement under ASC 808, with the related impact of the cost sharing included as research and development expense. All performance obligations were terminated upon the execution of the 2019 Casebia Agreements.

During the three months ended March 31, 2019, the Company recognized $0.2 million of revenue related to the collaboration with Casebia. During the three months ended March 31, 2019, the Company recognized $0.5 million of research and development expense related to the collaboration with Casebia. During the three months ended March 31, 2019, the Company recognized a loss from equity method investment of $1.0 million related to stock-based compensation expense for Casebia employees.

Accounting for the 2019 Casebia Agreements

The Company determined that the Retirement Agreement and Joint Venture Termination Agreement resulted in the Company obtaining a controlling interest in Casebia and should be accounted for as a separate component from the 2019 Option Agreement. In doing so, the Company allocated the consideration transferred of $41.0 million (consisting of $16.0 million of assets acquired net of the purchase price, as displayed in the table below, and $25.0 million of cash allocated to the 2019 Option Agreement) between the two components using a relative fair value approach. The Company determined the relative fair value related to obtaining a controlling interest in Casebia was $32.0 million and the relative fair value of the consideration transferred related to the 2019 Option Agreement was $25.0 million, which is comprised of $20.2 million related to certain research and development activities and $4.8 million related to certain options as described above.

As a result of the Retirement Agreement, the Company determined that it had obtained a controlling interest in a variable interest entity, for which it became the primary beneficiary. As such, under ASC 810, *Consolidation* , the Company accounted for the net assets obtained under ASC 805, *Business Combinations* . In accordance therewith, the Company determined the set of acquired assets and assumed liabilities did not meet the definition of a business, as the Company did not acquire an assembled workforce and thus the Company did not acquire substantive processes capable of producing outputs. As such, no goodwill was recorded. The Company measured the fair value of the assets and liabilities received, determining the relative fair value was $16.0 million (after paying the $16.0 million for Bayer's 50% interest) and recorded the difference between that amount and the Company's carrying amount, which was zero, as a gain within other income (expense). The relative fair value of the assets and liabilities received (exclusive of the $16.0 million paid from Casebia to Bayer to retire Bayer's interest in the JV) was determined as follows (in thousands):

Fair value	Amount
Cash and cash equivalents	$ 6,784
Prepaid expenses and other current assets	2,565
Property, plant and equipment, net	9,340
Operating lease assets	11,003
Restricted cash	1,226
Accrued expenses and other current liabilities	(3,915)
Operating lease liabilities	(11,003)
Net assets	$ 16,000

The value of the reacquired rights related to the intellectual property was determined to be insignificant.

The Company determined that the 2019 Option Agreement should be accounted for under ASC 730-20, *Research and Development Expense* . This determination was based on the fact that the financial risk associated with the research and development has been transferred to the Company because repayment of any of the funds provided by Bayer depends solely on the results of the research and development having a future economic benefit. The Company further determined that it had two separate obligations under the 2019 Option Agreements, which consist of research and development services and future delivery of up to two options for products in defined fields. The relative fair value of the obligations was determined to be $20.2 million and $4.8 million, respectively. As the Company has accounted for its obligations as a contract to perform research and development for others, with respect to the obligation to perform research and development services the Company will recognize an offset to research and development expense as the research is performed and, with respect to the future delivery of up to two option for products in defined fields, at the earlier of option exercise (at or near IND application filing), expiration, or when commercially reasonable efforts to progress the program have been exhausted.

During the three months ended March 31, 2020, the Company recorded a benefit of $1.9 million to research and development expense for qualifying expenses incurred under the 2019 Option Agreement. The Company has recorded $11.8 million in other current liabilities relating to certain research and development obligations to be satisfied within one year of the balance sheet date and $11.3 million in other long-term liabilities consisting of the relative fair value of such obligations to be satisfied beyond one year from the balance sheet date as well as the relative fair value of the options .

6. Share Capital

The Company had 103,901,006 authorized common shares as of March 31, 2020, with a par value of CHF 0.03 per share. Share Capital consisted of the following:

Type of Share Capital	Conditional Capital	As of March 31, 2020	As of December 31, 2019
Common shares	Registered share capital	61,036,566	61,036,566
Common shares	Authorized share capital	19,246,503	19,246,503
Common shares	Conditional share capital - Bonds or similar debt instruments	4,919,700	4,919,700
Common shares	Conditional share capital - Employee benefit plans	18,698,237	18,698,237
	Total	103,901,006	103,901,006

At-the-Market Offering

In August 2018, the Company entered into an Open Market Sale Agreement SM , or the 2018 ATM, with Jefferies LLC, or Jefferies, under which Jefferies was able to offer and sell, from time to time, common shares having aggregate gross proceeds of up to $125.0 million.

In August 2019, following the termination of the 2018 ATM by its terms, the Company entered into a new Open Market Sale Agreement SM with Jefferies, or the 2019 ATM, under which the Company may offer and sell, from time to time, common shares having aggregate gross proceeds of up to $200.0 million.

During the three months ended March 31, 2019, the Company sold 631,580 shares of common stock under the 2018 ATM for net cash proceeds of $23.9 million, after deducting commission fees of $0.7 million. For the year ended December 31, 2019, the Company issued and sold an aggregate of 2.8 million common shares under the 2018 ATM, at an average price of $44.38 per share, for aggregate proceeds of $120.6 million, which were net of equity issuance costs of $4.4 million.

As of March 31, 2020, the Company has not yet issued or sold any securities under the 2019 ATM.

7. Stock-based Compensation

During the three months ended March 31, 2020 and 2019, the Company recognized the following stock-based compensation expense (in thousands):

	Three Months Ended March 31, 2020	2019
Research and development	$ 7,362	$ 4,962
General and administrative	6,789	4,709
Loss from equity method investment	-	1,025
Total	$ 14,151	$ 10,696

Stock option activity

The following table summarizes stock option activity for the three months ended March 31, 2020:

	Shares (in thousands)	Weighted-average exercise price per share
Outstanding at December 31, 2019	7,782,437	$ 31.30
Granted	887,458	$ 45.73
Exercised	(88,466)	$ 19.37
Cancelled or forfeited	(86,114)	$ 42.20
Outstanding at March 31, 2020	8,495,315	$ 32.82
Exercisable at March 31, 2020	3,697,079	$ 25.25
Vested and expected to vest at March 31, 2020	8,495,315	$ 32.82

As of March 31, 2020, total unrecognized compensation expense related to stock options was $107.3 million, which the Company expects to recognize over a remaining weighted-average period of 2.8 years.

Restricted stock activity

The following table summarizes restricted stock activity for the three months ended March 31, 2020:

	Restricted Stock	Weighted-Average Grant Date Fair Value
Unvested balance as of December 31, 2019	699,534	$ 56.53
Granted	277,970	45.78
Vested	(5,000)	33.58
Cancelled or forfeited	(5,444)	51.18
Unvested balance as of March 31, 2020	967,060	$ 53.59

As of March 31, 2020, total unrecognized compensation expense related to unvested restricted common shares was $39.9 million, which the Company expects to recognize over a remaining weighted-average vesting period of 2.4 years.

8. Net Loss Per Share Attributable to Common Shareholders

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,	
	2020	2019
Outstanding options	8,495,315	8,032,582
Unvested restricted common shares	967,060	345,296
Total	9,462,375	8,377,878

9. Income Taxes

During the three months ended March 31, 2020 and 2019, the Company recorded an income tax provision of $0.4 million and $0.1 million, respectively, representing an effective tax rate of -0.5% and -0.2%, respectively. The income tax provision is primarily attributable to the year-to-date pre-tax income earned by the Company's U.S. subsidiary. The difference in the statutory tax rate and effective tax rate is primarily a result of the jurisdictional mix of earnings and losses that are not benefited. The Company maintains a valuation allowance against certain deferred tax assets that are not more-likely-than-not realizable. As a result, the Company has not recognized a tax benefit related to losses generated in Switzerland in the current periods. On March 27, 2020, the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, was enacted in the United States. The impact of the CARES Act on the Company for the period ending March 31, 2020 was not material.

10. Related Party Transactions

Casebia

Prior to the termination of the joint venture in December 2019, Casebia was a related party under ASC 850, *Related Party Disclosures* , or ASC 850 . Refer to Note 5, " *Joint Venture with Bayer Healthcare LLC.* "

Vertex

In the fourth quarter of 2018, upon becoming owners of record of more than 10% of the voting interest of the Company, Vertex became a related party under ASC 850 . As of July 2, 2019, upon becoming owners of record of less than 10% of the voting interest of the Company, Vertex was no longer a related party under ASC 850. Refer to Note 5, " *Agreements with Vertex Pharmaceuticals Incorporated and certain of its subsidiaries* ."

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, 2019 filed with the Securities and Exchange Commission, or the SEC, on February 12, 2020. 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 this Quarterly Report on Form 10-Q and the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2019, 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.

Special Note About Coronavirus (COVID-19)

In March 2020, we announced initial business impacts related to the outbreak of a novel strain of virus named SARS-CoV-2 (severe acute respiratory syndrome 2), or coronavirus, which causes coronavirus disease, or COVID-19. As a result of the coronavirus pandemic, we have experienced, and may further experience, disruptions that have and could further adversely impact our business operations as well as our preclinical studies and clinical trials. Specifically, while each of our Phase 1/2 open-label clinical trials of CTX001 for severe hemoglobinopathies (CLIMB THAL-111 and CLIMB SCD-121) are currently open for enrollment, since ICU beds and related healthcare resources are significantly constrained in light of the coronavirus pandemic, no additional patients are currently scheduled to begin dosing in either study at this time. In addition, with respect to our immuno-oncology clinical trials (which are also currently open for enrollment), investigators may not want to take the risk of exposing cancer patients to COVID-19 since the dosing of patients is conducted within an in-patient setting. In addition, certain aspects of our supply chain have been disrupted as certain of our third party suppliers and manufacturers have paused their operations in response to the coronavirus pandemic or have otherwise encountered delays in providing their services. We continue to evaluate the extent to which these pauses and delays will impact our ability to manufacture our product candidates for our clinical trials and conduct other research and development operations and maintain applicable timelines. The ultimate impact of the coronavirus pandemic on our business operations as well as our preclinical studies and clinical trials remains uncertain and subject to change and will depend on future developments, which cannot be accurately predicted. We will continue to monitor the situation closely.

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, who, along with 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 potentially treat a broad set of rare and common diseases by disrupting, correcting or regulating the genes related to such diseases. We believe that our scientific expertise, together with our approach, may enable an entirely new class of highly active and potentially curative therapies for patients for whom current biopharmaceutical approaches have had limited success.

We have established a portfolio of therapeutic programs across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine and rare diseases.

Our lead product candidate, CTX001, is an investigational, autologous, gene-edited hematopoietic stem cell therapy that is being evaluated for the treatment of transfusion-dependent beta thalassemia, or TDT, and severe sickle cell disease, or SCD. CTX001 is being developed under a co-development and co-commercialization agreement between us and Vertex.

We and Vertex are investigating CTX001 in a Phase 1/2 open-label clinical trial, CLIMB THAL-111, that is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 18 to 35 with TDT, non-beta zero/beta zero subtypes. The first two patients in the trial will be treated sequentially and, pending data from the initial two patients, the trial will open for broader concurrent enrollment. CLIMB THAL-111 is designed to enroll up to 45 patients and follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up study. CTX001 has been granted Fast Track Designation by the United States Federal Drug Administration, or FDA, for the treatment of TDT, as well as has been granted orphan drug designation, or ODD, by the European Commission. Enrollment is ongoing at multiple clinical trial sites globally. In the fourth quarter of 2019, we released preliminary clinical data from the first patient treated with CTX001 with TDT, and we expanded the TDT patient population for CTX001 to include beta zero/beta zero subtypes.

We and Vertex are also investigating CTX001 in a Phase 1/2 open-label clinical trial, CLIMB SCD-121, that is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 18 to 35 with severe SCD. Similar to the trial in beta thalassemia, the first two patients in the trial will be treated sequentially and, pending data from the initial two patients, the trial will open for broader concurrent enrollment. CLIMB SCD-121 is designed to enroll up to 45 patients and follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up study. CTX001 has been granted Fast Track Designation by the FDA for the treatment of SCD, as well as ODD by the European Commission. Enrollment is ongoing at multiple clinical trial sites globally. In the fourth quarter of 2019, we released preliminary clinical data from the first patient treated with CTX001 with severe SCD.

In addition, we are developing our own portfolio of CAR-T cell product candidates based on our gene-editing technology.

CTX110 . Our lead candidate, CTX110, is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting cluster differentiation 19, or CD19, for the treatment of CD19+ malignancies. CTX110 is being investigated in a Phase 1 clinical trial that is designed to assess the safety and efficacy of CTX110 for the treatment of relapsed or refractory B-cell malignancies. The multi-center, open-label clinical trial is designed to enroll up to 95 patients and investigate several dose levels of CTX110. The trial is currently enrolling at multiple clinical trial sites globally.

CTX120 . CTX120 is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting B-cell maturation antigen. CTX120 is being investigated in a Phase 1 clinical trial that is designed to assess the safety and efficacy of CTX120 for the treatment of relapsed or refractory multiple myeloma. The multi-center, open-label clinical trial is designed to enroll up to 80 patients and investigate several dose levels of CTX120. The trial is currently enrolling.

CTX130 . CTX130 is a healthy donor-derived gene-edited allogeneic CAR-T therapy targeting cluster of differentiation 70, or CD70, an antigen expressed on various solid tumors and hematologic malignancies. CTX130 is being developed for the treatment of both solid tumors, such as renal cell carcinoma, and T-cell and B-cell hematologic malignancies.

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 three broad strategic partnerships to develop gene editing-based therapeutics in specific disease areas.

Vertex . We established our initial collaboration agreement in 2015 with Vertex Pharmaceuticals Incorporated and certain subsidiaries, or 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 to co-develop and co-commercialize CTX001 as part of that collaboration. In June 2019, we expanded our collaboration and entered into a strategic collaboration and license agreement for the development and commercialization of products for the treatment of Duchenne muscular dystrophy and myotonic dystrophy type 1 .

ViaCyte . We entered into the ViaCyte Collaboration Agreement in September 2018 with ViaCyte, Inc., or ViaCyte, to pursue the discovery, development and commercialization of gene-edited allogeneic stem cell therapies for the treatment of diabetes. The combination of ViaCyte's stem cell capabilities and our gene-editing capabilities has the potential to enable a beta-cell replacement product that may deliver durable benefit to patients without the need for immune suppression.

Bayer . In the fourth quarter of 2019, we entered into a series of transactions, or the Bayer Transaction, pursuant to which we and Bayer terminated our 2015 agreement, which created the joint venture, Casebia Therapeutics Limited Liability Partnership, or Casebia, to discover, develop and commercialize CRISPR/Cas9 gene-editing therapeutics to treat the genetic causes of bleeding disorders, autoimmune disease, blindness, hearing loss and heart disease. In connection thereto, Casebia became a wholly-owned subsidiary of ours. We and Bayer also entered into a new option agreement pursuant to which Bayer has an option to co-develop and co-commercialize two products 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.

Refer to Note 7 of the notes to our consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2019 for a description of the key terms of our arrangement with ViaCyte. Refer to Note 5 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for a description of the key terms of our arrangements with Vertex and Bayer.

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.

All of our revenue to date has been collaboration revenue. We were profitable for the year ended December 31, 2019 due to collaboration revenue from Vertex and Casebia, but we do not expect to sustain our profitability in future years. With the exception of the year ended December 31, 2019, we have incurred significant net operating losses each year since our inception and expect to

continue to incur net operating losses for the foreseeable future. As of March 31, 2020, we had $ 889.7 million in cash and cash equivalents and an accumulated deficit of $2 9 4 . 4 million . We expect to continue to incur significant expenses and increasing operating losses for the next several years. 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; maintain, expand and protect our intellectual property estate; further develop our gene editing platform; hire additional research, clinical and scientific personnel; and incur additional costs associated with operating as a public company.

Financial Overview

Revenue

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, 2020 and 2019, respectively, 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, 2019 filed with the SEC on February 12, 2020, as well as Note 5 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 activities on our behalf;
- costs of purchasing lab supplies and non-capital equipment used in our preclinical activities and in manufacturing preclinical 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.

R esearch 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.

Except for activities we perform in connection with our collaboration with Vertex, as well as certain arrangements we had with Casebia prior to the Bayer Transaction, we do not track research and development costs on a program-by-program basis.

Research and development activities are central to our business model. We expect our research and development costs to increase significantly for the foreseeable future as our current development programs progress, new programs are added and as 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. In addition, we expect that our research and development expenses will increase in future periods as we incur additional costs in connection with research and development activities under our collaboration with ViaCyte.

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 and human resources 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 anticipate that our general and administrative expenses will increase in the future to support continued research and development activities, potential commercialization of our product candidates and increased costs of operating as a public company. In addition, we anticipate increased expenses related to the reimbursements of third-party patent related expenses in connection with certain of our in-licensed intellectual property.

Results of Operations

Comparison of three months ended March 31, 2020 and 2019 (in thousands):

	Three Months Ended March 31,		Period to Period Change
	2020	2019	
Collaboration revenue	$ 157	$ 328	$ (171)
Operating expenses:			
Research and development	54,193	$ 33,822	20,371
General and administrative	19,550	$ 14,929	4,621
Total operating expenses	73,743	48,751	24,992
Loss from operations	(73,586)	(48,423)	(25,163)
Other income, net	4,232	$ 100	4,132
Net loss before income taxes	(69,354)	(48,323)	(21,031)
Provision for income taxes	(377)	$ (85)	(292)
Net loss	$ (69,731)	$ (48,408)	$ (21,323)

Collaboration Revenue

Collaboration revenue for the three months ended March 31, 2020 and 2019, respectively, was not material. Please refer to Note 5 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 $54.2 million for the three months ended March 31, 2020, compared to $33.8 million for the three months ended March 31, 2019. The increase of approximately $20.4 million was primarily attributable to the following:

- $7.5 million of increased employee compensation, benefit and other headcount related expenses, of which $2.4 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth;
- $7.5 million of increased facility-related expenses;
- $3.9 million of increased variable research and development costs; and,
- $1.5 million of increased license fees.

General and Administrative Expenses

General and administrative expenses were $19.5 million for the three months ended March 31, 2020, compared to $14.9 million for the three months ended March 31, 2019. The increase of approximately $4.6 million was primarily attributable to the following:

- $3.2 million of increased employee compensation, benefit and other headcount related expenses, of which $2.1 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth; and,
- $1.2 million of increased facility-related expenses.

Other Income, Net

Other income was $4.2 million for the three months ended March 31, 2020, compared to $0.1 million of income for the three months ended March 31, 2019. The change was primarily due to interest income earned on cash and cash equivalents for the three months ended March 31, 2020.

Liquidity and Capital Resources

As of March 31, 2020, we had cash and cash equivalents of approximately $889.7 million, of which approximately $840.8 million was held outside of the United States. In April 2020, we received a payment of $25.0 million in connection with the 2019 collaboration agreement with Vertex. Refer to Note 5, “Significant Contracts” in our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for a discussion.

We have predominantly incurred losses and cumulative negative cash flows from operations since our inception, and as of March 31, 2020, we had an accumulated deficit of $294.4 million. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase 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.

Funding Requirements

Our primary uses of capital are, and we expect will continue to be, research and development activities, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses, patent prosecution filing and maintenance costs for our licensed intellectual property and general overhead costs. We expect our expenses to increase compared to prior periods in connection with our ongoing activities, particularly as we continue research and development and preclinical activities and initiate preclinical studies to support initial drug applications. In addition, we expect to incur additional costs associated with operating as a public company.

Because our research 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 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, debt financings and payments received in connection with our collaboration agreements. We are entitled to research payments under our collaboration with Vertex. Additionally, we are eligible to earn payments, in each case, on a per-product basis under our collaboration with Vertex. Except for this source of funding, we do not have any committed external source of liquidity. 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, as a result of the coronavirus pandemic, t he 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 resulting from the spread of the coronavirus 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, 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
	2020	2019	
Net cash used in operating activities	$ (52,175)	$ (43,737)	$ (8,438)
Net cash used in investing activities	(2,991)	(1,097)	(1,894)
Net cash provided by financing activities	1,132	25,726	(24,594)
Effect of exchange rate changes on cash	(25)	8	(33)
Net decrease in cash	$ (54,059)	$ (19,100)	$ (34,959)

Net Cash Used in Operating Activities

Net cash used in operating activities was $52.2 million for the three months ended March 31, 2020, compared to cash used of $43.7 million for the three months ended March 31, 2019. The $8.4 million increase in cash used in operating activities was primarily driven by the increase in net loss of $21.3 million, which was driven by increased spending on our clinical and pre-clinical stage programs and increased payroll and payroll-related expenses to support overall growth. The increase was offset by an increase of $5.6 million of non-cash expense and an increase of $7.3 in net changes of operating assets and liabilities.

Net Cash Used in Investing Activities

Net cash used in investing activities for the three months ended March 31, 2020 was $3.0 million, compared to $1.1 million for the three months ended March 31, 2019. The increase in net cash used in investing activities consisted primarily of purchases of property and equipment.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the three months ended March 31, 2020 was $1.1 million, compared with $25.7 million for the three months ended March 31, 2019. The net cash provided by financing activities for the three months ended March 31, 2020 consisted of option exercise proceeds, net of issuance costs. The net cash provided by financing activities for the three months ended March 31, 2019 consisted of proceeds from the issuance of common shares in connection with an at-the-market sales agreement with Jefferies LLC, which resulted in $23.9 million of net cash proceeds, after deducting $0.7 million in commissions, as well as exercises of stock options.

Contractual Obligations

The disclosure of our contractual obligations and commitments was reported in our Annual Report on Form 10-K for the year ended December 31, 2019 filed with the SEC on February 12, 2020. There have been no material changes from the contractual commitments and obligations previously disclosed in our Annual Report on Form 10-K.

Off-Balance Sheet Arrangements

As of March 31, 2020, we did not have any off-balance sheet arrangements as defined under applicable SEC rules.

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, variable interest entities 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, 2019 filed with the SEC on February 12, 2020.

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

Cash and cash equivalents were held primarily in cash deposits and money market funds. The fair value of our cash and cash equivalents would not be significantly affected by either an increase or decrease in interest rates due mainly to the short-term nature of these instruments.

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.

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, 2020 , 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, 2020 , 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, 2020, 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.

From time to time, we may become involved in litigation or other legal proceedings relating to claims arising from the ordinary course of business. 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, 2019 filed with the SEC on February 12, 2020.

Item 1A. Risk Factors.

In light of the rapid spread of SARS-CoV-2, which causes coronavirus disease 2019, or COVID-19, in the United States and globally, 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, 2019 filed with the SEC on February 12, 2020 to add the following new risk factor:

Our business may be adversely affected by the ongoing coronavirus pandemic.

Our business could be adversely affected by health epidemics in regions where we have concentrations of clinical trial sites or other business activities and could cause significant disruption in the operations of third party manufacturers and CROs upon whom we rely. For example, beginning in late 2019, the outbreak of a novel strain of virus named SARS-CoV-2 (severe acute respiratory syndrome coronavirus 2), or coronavirus, which causes coronavirus disease 2019, or COVID-19, has evolved into a global pandemic. As of late March 2020, the coronavirus had spread to most regions of the world.

As a result of the coronavirus pandemic, we have experienced and may further experience disruptions that have and could further impact our business, preclinical studies and clinical trials, including:

- We are conducting a number of clinical trials for product candidates in the fields of severe hemoglobinopathies and immuno-oncology in geographies which are affected by the coronavirus pandemic. We believe that the coronavirus pandemic has had, and will likely continue to have, an impact on various aspects of our clinical trials. For example, with respect to our CTX001 clinical trials for severe hemoglobinopathies (specifically, transfusion-dependent beta thalassemia and severe sickle cell disease), since ICU beds and related healthcare resources are anticipated to become significantly constrained in light of the coronavirus pandemic, no additional patients are currently scheduled to begin dosing in either study at this time. And, for example, with respect to our immuno-oncology clinical trials, investigators may not want to take the risk of exposing cancer patients to COVID-19 since the dosing of patients is conducted within an in-patient setting. Other potential impacts of the coronavirus pandemic on our various clinical trials include patient dosing and study monitoring, which may be paused or delayed due to changes in policies at various clinical sites, federal, state, local or foreign laws, rules and regulations, including quarantines or other travel restrictions, prioritization of healthcare resources toward pandemic efforts, including diminished attention of physicians serving as our clinical trial investigators and reduced availability of site staff supporting the conduct of our clinical trials, interruption or delays in the operations of the U.S. Food and Drug Administration, or other reasons related to the coronavirus pandemic. If the coronavirus pandemic continues, other aspects of our clinical trials may be adversely affected, delayed or interrupted, including, for example, site initiation, patient recruitment and enrollment, availability of clinical trial materials, and data analysis. Some patients and clinical investigators may not be able to comply with clinical trial protocols and patients may choose to withdraw from our studies or we may have to pause enrollment or we may choose to or be required to pause enrollment and or patient dosing in our ongoing clinical trials in order to preserve health resources and protect trial participants. It is unknown how long these pauses or disruptions could continue.
- We currently rely on third parties to, among other things, manufacture raw materials, manufacture our product candidates for our clinical trials, shipping of investigational drugs and clinical trial samples, perform quality testing and supply other goods and services to run our business. Certain of our third party manufacturers and suppliers have paused their operations in response to the coronavirus pandemic or have otherwise encountered delays in providing their services. As a result, we may not be able to manufacture our product candidates for our clinical trials and conduct other research and development operations and maintain current clinical and pre-clinical timelines. In addition, if additional third parties in our supply chain for materials are adversely impacted by restrictions resulting from the coronavirus pandemic, including staffing shortages, production slowdowns and disruptions in delivery systems, our supply chain may be disrupted in other ways, further limiting our ability to manufacture our product candidates for our clinical trials and conduct our research and development operations.

- We have closed our offices and requested that most of our personnel, including all of our administrative employees, to work remotely, restricted on-site staff to only those personnel and contractors who must perform essential activities that must be completed on-site and limited the number of staff in any given research and development laboratory. Our increased reliance on personnel working from home may negatively impact productivity, or disrupt, delay, or otherwise adversely impact our business. In addition, this could increase our cyber security risk, create data accessibility concerns, and make us more susceptible to communication disruptions, any of which could adversely impact our business operations or delay necessary interactions with local and federal regulators, ethics committees, manufacturing sites, research or clinical trial sites and other important agencies and contractors.
- Our employees and contractors conducting research and development activities may not be able to access our laboratory for an extended period of time as a result of the closure of our offices and the possibility that governmental authorities further modify current restrictions now, or in the future, if additional waves of coronavirus infections were to occur. As a result, this could delay timely completion of preclinical activities, including completing Investigational New Drug (IND)/Clinical Trial Application (CTA)-enabling studies or our ability to select future development candidates, and initiation of additional clinical trials for other of our development programs. In addition, when we re-open our facilities, we could encounter delays in connection with implementing precautionary measures to mitigate the risk of exposing our facilities and employees to the coronavirus (for example, implementing screening procedures or procuring appropriate non-medical personal protective equipment for use while in our facilities) or otherwise in connection with addressing an actual or potential exposure to the coronavirus (for example, temporarily closing all or a portion of a facility or disinfecting all or a portion of a facility that may have been exposed to the coronavirus).
- Health regulatory agencies globally may experience disruptions in their operations as a result of the coronavirus pandemic. The U.S. Food and Drug Administration, or FDA, and comparable foreign regulatory agencies may have slower response times or be under-resourced to continue to monitor our clinical trials and, as a result, review, inspection, and other timelines may be materially delayed. It is unknown how long these disruptions could continue, were they to occur. Any elongation or de-prioritization of our clinical trials or delay in regulatory review resulting from such disruptions could materially affect the development and study of our product candidates. For example, regulatory authorities may require that we not distribute a product candidate lot until the relevant agency authorizes its release. Such release authorization may be delayed as a result of the coronavirus pandemic and could result in delays to our clinical trials.
- The trading prices for our common shares and other biopharmaceutical companies have been highly volatile as a result of the coronavirus pandemic. 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 resulting from the spread of the coronavirus could materially and adversely affect our business and the value of our common shares.

The coronavirus pandemic continues to rapidly evolve. The ultimate impact of the coronavirus pandemic on our business operations is highly uncertain and subject to change and will depend on future developments, which cannot be accurately predicted, including the duration of the pandemic, additional or modified government actions, new information that will emerge concerning the severity and impact of COVID-19 and the actions taken to contain coronavirus or address its impact in the short and long term, among others. We do not yet know the full extent of potential delays or impacts on our business, our clinical trials, our research programs, healthcare systems or the global economy. We will continue to monitor the situation closely.

There have been no other material changes from the risk factors disclosed in our Annual Report on Form 10-K filed with the SEC on February 12, 2020. 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.

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

On February 25, 2020, we entered into a License Agreement and Share Purchase Agreement with a private company pursuant to which we in-licensed certain technology and, on March 11, 2020, issued to such company 17,830 common shares as consideration for such in-license. The offer, sale, and issuance of the shares is exempt from registration pursuant to Section 4(a)(2) of the Securities Act of 1933, as amended, as a transaction by an issuer not involving a public offering.

Item 5 . Other Information

As disclosed in Note 5 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q and Part I, Item 2, Management's Discussion and Analysis of Financial Condition and Results of Operations, we received $25.0 million from Vertex in April 2020 in connection with achieving the first milestone under the 2019 collaboration agreement in the first quarter of 2020.

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
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.

\+ 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: April 28, 2020

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

Dated: April 28, 2020

By: /s/ Michael Tomsicek
Michael Tomsicek
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: April 28, 2020

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

Exhibit 31.2

CERTIFICATIONS

I, Michael Tomsicek, 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: April 28, 2020

By: /s/ Michael Tomsicek
Michael Tomsicek
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, 2020 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)

April 28, 2020

/s/ Michael Tomsicek
Michael Tomsicek
Chief Financial Officer
(Principal Financial and Accounting Officer)

April 28, 2020