

**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 September 30, 2019**

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
(State or other jurisdiction of
incorporation or organization)

Not Applicable
(I.R.S. Employer
Identification No.)

Baarerstrasse 14
6300 Zug, Switzerland
(Address of principal executive offices)

Not Applicable
(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	NASDAQ Stock Market LLC

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

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

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

As of October 24, 2019, there were 55,204,242 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.

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,” “project,” “will,” “would” or the negative or plural of these words or similar expressions or variations, although not all forward-looking statements contain these identifying words. Forward-looking statements in this Quarterly Report on Form 10-Q include, but are not limited to, statements about:

- the safety, efficacy and clinical progress of our various clinical programs including CTX001® and CTX110™;
- the status of clinical trials, development timelines and discussions with regulatory authorities related to product candidates under development by us and our collaborators;
- the proposed transaction involving Casebia Therapeutics;
- 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;
- 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, and in other Securities and Exchange Commission (“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.

Index

	<u>Page Number</u>
<u>PART I: FINANCIAL INFORMATION</u>	
<u>Item 1. Condensed Consolidated Financial Statements (unaudited)</u>	
<u>Condensed Consolidated Balance Sheets as of September 30, 2019 and December 31, 2018</u>	2
<u>Condensed Consolidated Statements of Operations and Comprehensive Income (Loss) for the three and nine months ended September 30, 2019 and 2018</u>	3
<u>Condensed Consolidated Statements of Shareholders' Equity for the three and nine months ended September 30, 2019 and 2018</u>	4
<u>Condensed Consolidated Statements of Cash Flows for the nine months ended September 30, 2019 and 2018</u>	5
<u>Notes to Condensed Consolidated Financial Statements</u>	6
<u>Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	19
<u>Item 3. Quantitative and Qualitative Disclosures about Market Risk</u>	26
<u>Item 4. Controls and Procedures</u>	26
<u>PART II: OTHER INFORMATION</u>	
<u>Item 1. Legal Proceedings</u>	27
<u>Item 6. Exhibits</u>	28
<u>SIGNATURES</u>	29

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	
	September 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	\$ 629,717	\$ 456,649
Accounts receivable, including related party amounts of \$ 39 and \$88 as of September 30, 2019 and December 31, 2018, respectively	39	88
Prepaid expenses and other current assets, including related party amounts of \$ 28,875 and \$3,417 as of September 30, 2019 and December 31, 2018, respectively	34,309	9,658
Total current assets	664,065	466,395
Property and equipment, net	21,074	18,500
Intangible assets, net	249	289
Restricted cash	3,915	3,163
Operating lease assets	30,619	—
Other non-current assets	668	669
Total assets	<u>\$ 720,590</u>	<u>\$ 489,016</u>
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	\$ 3,374	\$ 5,069
Accrued expenses, including related party amounts of \$ 4,537 and \$ 1,700 as of September 30, 2019 and December 31, 2018, respectively	22,397	20,852
Deferred revenue current, including related party amounts of \$ 47,725 and \$ 102 as of September 30, 2019 and December 31, 2018, respectively	47,725	102
Accrued tax liabilities	1,478	402
Deferred rent	—	1,202
Operating lease liabilities	4,805	—
Other current liabilities	—	119
Total current liabilities	79,779	27,746
Deferred revenue non-current, including related party amounts of \$ 11,871 and \$ 57,780 as of September 30, 2019 and December 31, 2018, respectively	11,871	57,780
Deferred rent non-current	—	11,052
Operating lease liabilities, net of current portion	36,827	—
Other non-current liabilities	235	243
Total liabilities	<u>128,712</u>	<u>96,821</u>
Commitments and contingencies, see Note 4		
Shareholders' equity:		
Common shares, CHF 0.03 par value, 55,677,088 and 52,183,139 shares authorized at September 30, 2019 and December 31, 2018, respectively, 55,446,359 and 52,160,798 shares issued at September 30, 2019 and December 31, 2018, respectively, 51,852,862 shares outstanding at September 30, 2019 and December 31, 2018, respectively, 23,948,128 and 20,498,996 shares in conditional capital at September 30, 2019 and December 31, 2018, respectively.	1,684	1,584
Treasury shares, at cost, 256,989 and 307,936 shares at September 30, 2019 and December 31, 2018, respectively	(57)	(57)
Additional paid-in capital	845,526	682,245
Accumulated deficit	(255,253)	(291,569)
Accumulated other comprehensive loss	(22)	(8)
Total shareholders' equity	<u>591,878</u>	<u>392,195</u>
Total liabilities and shareholders' equity	<u>\$ 720,590</u>	<u>\$ 489,016</u>

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Collaboration revenue (1)	\$ 211,928	\$ 563	\$ 212,574	\$ 3,009
Operating expenses:				
Research and development (2)	57,246	39,820	130,601	84,972
General and administrative	15,519	10,175	46,216	31,752
Total operating expenses	72,765	49,995	176,817	116,724
Income (loss) from operations	139,163	(49,432)	35,757	(113,715)
Other income (expense):				
Loss from equity method investment	(3,430)	(1,012)	(5,467)	(3,256)
Other income (expense), net	2,964	(130)	6,470	(101)
Total other income (expense), net	(466)	(1,142)	1,003	(3,357)
Net income (loss) before income taxes	138,697	(50,574)	36,760	(117,072)
Provision for income taxes	(274)	(137)	(444)	(319)
Net income (loss)	138,423	(50,711)	36,316	(117,391)
Foreign currency translation adjustment	(12)	(6)	(14)	(15)
Comprehensive income (loss)	\$ 138,411	\$ (50,717)	\$ 36,302	\$ (117,406)
Reconciliation of net income (loss) to net income (loss) attributable to common shareholders:				
Net income (loss)	\$ 138,423	\$ (50,711)	\$ 36,316	\$ (117,391)
Net income (loss) per share attributable to common shareholders—basic	\$ 2.52	\$ (1.07)	\$ 0.68	\$ (2.51)
Weighted-average common shares outstanding used in net income (loss) per share attributable to common shareholders—basic	54,829,057	47,391,988	53,380,123	46,709,388
Net income (loss) per share attributable to common shareholders—diluted	\$ 2.40	\$ (1.07)	\$ 0.65	\$ (2.51)
Weighted-average common shares outstanding used in net income (loss) per share attributable to common shareholders—diluted	57,598,901	47,391,988	55,821,420	46,709,388
(1) Including the following revenue from a related party, see Notes 6 & 11:	\$ 211,928	\$ 443	\$ 212,574	\$ 2,406
(2) Including the following research and development expense with a related party, see Notes 6 & 11:	\$ 17,563	\$ 418	\$ 32,022	\$ 2,770

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		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Shareholders' Equity
	Shares	CHF 0.03 Par Value	Shares	Amount, at cost				
Balance at December 31, 2017	40,592,248	1,240	444,873	—	312,018	(125,440)	14	187,832
Cumulative effect of ASC 606 adoption	—	—	—	—	—	(1,148)	—	(1,148)
Issuance of common shares, net of issuance costs of \$ 8.2 million	5,750,000	174	—	—	122,423	—	—	122,597
Vesting of restricted shares	10,042	—	—	—	13	—	—	13
Exercise of vested options	328,525	9	(6,253)	—	2,647	—	—	2,656
Stock-based compensation expense	—	—	—	—	6,673	—	—	6,673
Other comprehensive loss	—	—	—	—	—	—	12	12
Net loss	—	—	—	—	—	(28,300)	—	(28,300)
Balance at March 31, 2018	46,680,815	\$ 1,423	438,620	\$ —	\$ 443,774	\$ (154,888)	\$ 26	\$ 290,335
Issuance of common shares, net of issuance costs of \$ 0.0M	—	—	—	—	—	—	—	—
Vesting of restricted shares	10,043	1	—	—	13	—	—	14
Exercise of vested options	380,977	11	(29,259)	(50)	3,768	—	—	3,729
Stock-based compensation expense	—	—	—	—	9,477	—	—	9,477
Other Comprehensive loss	—	—	—	—	—	—	(21)	(21)
Net loss	—	—	—	—	—	(38,380)	—	(38,380)
Balance at June 30, 2018	47,071,835	\$ 1,435	409,361	\$ (50)	\$ 457,032	\$ (193,268)	\$ 5	\$ 265,154
Issuance of common shares, net of issuance costs of \$ 15.5M	4,210,526	137	—	—	184,319	—	—	184,456
Vesting of restricted shares	10,046	1	—	—	20	—	—	21
Exercise of vested options	161,388	2	(741)	—	1,839	—	—	1,841
Repurchase of treasury shares	(64,952)	—	64,952	(7)	—	—	—	(7)
Issuance of treasury shares to ViaCyte	165,636	—	(165,636)	—	7,500	—	—	7,500
Stock-based compensation expense	—	—	—	—	9,066	—	—	9,066
Other Comprehensive loss	—	—	—	—	—	—	(6)	(6)
Net loss	—	—	—	—	—	(50,711)	—	(50,711)
Balance at September 30, 2018	51,554,479	\$ 1,575	307,936	\$ (57)	\$ 659,776	\$ (243,979)	\$ (1)	\$ 417,314
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 loss	—	—	—	—	—	—	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
Issuance of common shares, net of issuance costs of \$ 1.3 million	732,108	40	(47,297)	—	28,074	—	—	28,114
Vesting of restricted shares	12,317	1	—	—	15	—	—	16
Exercise of vested options	118,987	—	(3,650)	—	1,254	—	—	1,254
Stock-based compensation expense	—	—	—	—	12,198	—	—	12,198
Other comprehensive loss	—	—	—	—	—	—	(10)	(10)
Net loss	—	—	—	—	—	(53,699)	—	(53,699)
Balance at June 30, 2019	53,499,057	\$ 1,630	256,989	\$ (57)	\$ 759,796	\$ (393,676)	\$ (10)	\$ 367,683
Issuance of common shares, net of issuance costs of \$ 2.3 million	1,452,880	43	—	—	68,618	—	—	68,661
Vesting of restricted shares	34,328	1	—	—	11	—	—	12
Exercise of vested options	203,105	10	—	—	2,328	—	—	2,338
Stock-based compensation expense	—	—	—	—	14,773	—	—	14,773
Other comprehensive loss	—	—	—	—	—	—	(12)	(12)
Net loss	—	—	—	—	—	138,423	—	138,423
Balance at September 30, 2019	55,189,370	\$ 1,684	256,989	\$ (57)	\$ 845,526	\$ (255,253)	\$ (22)	\$ 591,878

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)

	<u>Nine Months Ended September 30,</u>	
	<u>2019</u>	<u>2018</u>
Operating activities:		
Net income (loss)	\$ 36,316	\$ (117,391)
Reconciliation of net income (loss) to net cash used in operating activities:		
Depreciation and amortization	3,293	2,574
Equity-based compensation	32,200	21,960
Loss from equity method investment	5,467	3,256
Expense related to ViaCyte transaction	—	15,109
Other income, non-cash	—	(169)
Changes in:		
Accounts receivable	49	2,074
Prepaid expenses and other assets	(25,342)	(2,502)
Accounts payable and accrued expenses	420	7,982
Deferred revenue	1,714	(270)
Deferred rent	—	(528)
Operating lease assets and liabilities	(689)	—
Other liabilities, net	(127)	38
Net cash provided by (used in) operating activities	<u>53,301</u>	<u>(67,867)</u>
Investing activities:		
Purchase of property and equipment	(5,732)	(1,773)
Net cash used in investing activities	<u>(5,732)</u>	<u>(1,773)</u>
Financing activities:		
Proceeds from issuance of common shares, net of issuance costs	121,216	309,019
Proceeds from exercise of options	5,049	8,241
Repurchase of treasury shares	—	(57)
Net cash provided by financing activities	<u>126,265</u>	<u>317,203</u>
Effect of exchange rate changes on cash	(14)	(15)
Increase in cash	<u>173,820</u>	<u>247,548</u>
Cash, cash equivalents and restricted cash, beginning of period	<u>459,812</u>	<u>242,912</u>
Cash, cash equivalents and restricted cash, end of period	<u>\$ 633,632</u>	<u>\$ 490,460</u>
Supplemental disclosure of non-cash investing and financing activities		
Property and equipment purchases in accounts payable and accrued expenses	\$ 428	\$ —
Equity issuance costs in accounts payable and accrued expenses	\$ 739	\$ 1,980
Reconciliation to amounts within the condensed consolidated balance sheets		
	<u>As of September 30,</u>	
	<u>2019</u>	<u>2018</u>
Cash and cash equivalents	\$ 629,717	\$ 487,295
Restricted cash	3,915	3,165
Cash, cash equivalents and restricted cash at end of period	<u>633,632</u>	<u>490,460</u>

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

CRISPR Therapeutics AG
Notes to Condensed Consolidated Financial Statements
(unaudited)

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 (“GAAP”).

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. The Company views its operations and manages its business in one operating segment, which is the business of discovering, developing and commercializing therapies derived from or incorporating genome-editing technology. Certain information and footnote disclosures normally included in the Company’s annual financial statements have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of operations for the three and nine month interim periods ended September 30, 2019 and 2018.

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, 2018, which are contained in the 2018 Annual Report on Form 10-K filed with the Securities and Exchange Commission (“SEC”) on February 25, 2019.

Certain items in the prior year’s condensed consolidated financial statements have been reclassified to conform to the current presentation. As a result, no subtotals in the prior year condensed consolidated financial statements were impacted.

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. Significant estimates in these consolidated financial statements have been made in connection with the calculation of revenues, research and development expenses and equity-based compensation expense. The Company bases its estimates on historical experience and various other assumptions, including, in certain circumstances, future projections that management believes to be reasonable. Actual results could differ from those estimates. 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 and nine months ended September 30, 2019 are consistent with those discussed in Note 2 to the consolidated financial statements in the Company’s 2018 Annual Report on Form 10-K filed with the SEC on February 25, 2019, except with respect to the Company’s lease accounting policy noted within the “Recently adopted accounting standards” section below.

Recently Adopted Accounting Standards

The Company adopted ASC 842, *Leases* (“ASC 842”), using the required modified retrospective approach, effective January 1, 2019. The Company chose to apply the transition provisions as of the period of adoption. The Company elected the package of practical expedients permitted under the transition guidance within the new standard, which, among other things, allowed the Company to carry forward the historical lease classification. In addition, the Company elected the practical expedient not to apply the recognition requirements in the lease standard to short-term leases (a lease that at commencement date has a lease term of 12 months or less and does not contain a purchase option that it is reasonably certain to exercise) and the practical expedient that permits lessees to make an accounting policy election (by class of underlying asset) to account for each separate lease component of a contract and its associated non-lease components as a single lease component. The adoption of the new standard resulted in the recording net lease assets and lease liabilities of \$26.1 million and \$37.6 million, respectively, as of January 1, 2019. The difference between the additional lease assets and lease liabilities is primarily due to the change in classification of lease incentives from liabilities to a reduction in our net lease assets. The standard had no impact on our net loss or cash flows.

Impact of Adopting ASC 842 on the Financial Statements

	January 1, 2019 Prior to ASC 842 Adoption	ASC 842 Adjustment	January 1, 2019 As Adjusted
Consolidated Balance Sheet Data (in thousands):			
Prepaid expenses and other current assets ⁽¹⁾	\$ 9,658	\$ (553)	\$ 9,105
Operating lease assets ⁽²⁾	\$ —	\$ 26,087	\$ 26,087
Deferred rent ⁽³⁾⁽⁴⁾	\$ 1,026	\$ (1,026)	\$ —
Deferred rent non-current ⁽³⁾	\$ 11,052	\$ (11,052)	\$ —
Operating lease liabilities ⁽⁵⁾	\$ —	\$ 4,930	\$ 4,930
Non-current operating lease liabilities ⁽⁵⁾	\$ —	\$ 32,682	\$ 32,682

(1) Represents reclassification of prepaid rent to operating lease assets.

(2) Represents capitalization of operating lease assets and reclassification of equipment licenses from prepaid expenses to operating lease assets, offset by reclassification of deferred rent to operating lease assets.

(3) Represents reclassification of deferred rent and tenant incentives to operating lease assets.

(4) As of December 31, 2018, the deferred rent balance was \$1,202, which included \$176 of sublease income received prior to year-end but not due until January 1, 2019.

(5) Represents recognition of operating lease liabilities.

2. Property and Equipment, net

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

	As of	
	September 30, 2019	December 31, 2018
Computer equipment	\$ 585	\$ 443
Furniture, fixtures and other	2,640	2,453
Laboratory equipment	12,212	8,964
Leasehold improvements	16,210	13,776
Construction work in process	55	239
Total property and equipment, gross	31,702	25,875
Accumulated depreciation	(10,628)	(7,375)
Total property and equipment, net	<u>\$ 21,074</u>	<u>\$ 18,500</u>

Depreciation expense for the three and nine months ended September 30, 2019 was \$1.3 million and \$3.3 million, respectively. Depreciation expense for the three and nine months ended September 30, 2018 was \$0.8 million and \$2.6 million, respectively.

3. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	As of	
	September 30, 2019	December 31, 2018
Payroll and employee-related costs	\$ 8,174	\$ 7,321
Research costs	9,853	7,973
Licensing fees	26	625
Professional fees	2,537	1,848
Intellectual property costs	1,739	2,193
Accrued property and equipment	53	294
Other	15	598
Total	<u>\$ 22,397</u>	<u>\$ 20,852</u>

4. Commitments and Contingencies

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.

As disclosed in its Current Report on Form 8-K filed with the SEC on June 26, 2019, on June 25, 2019, the Company received notification that the United States Patent and Trademark Office initiated an interference proceeding at the Patent Trial and Appeal Board (the "PTAB") between certain pending U.S. patent applications co-owned by the University of California, the University of Vienna and Dr. Emmanuelle Charpentier (collectively, the "CVC Group") and certain patents and a patent application currently owned by the Broad Institute and Massachusetts Institute of Technology and, in some instances, the President and Fellows of Harvard College (individually and collectively, the "Broad"), all of which are related to the single guide format of CRISPR/Cas9 genome editing technology in eukaryotic cells. The Company has an exclusive worldwide license in the field of human therapeutics to Dr. Charpentier's rights as a co-owner of the CVC Group portfolio. Specifically, the PTAB has declared Patent Interference No. 106,115 between the CVC Group's pending U.S. Patent Application Nos. 15/947,680; 15/947,700; 15/947,718; 15/981,807; 15/981,808; 15/981,809; 16/136,159; 16/136,165; 16/136,168; and 16/136,175, and the Broad's U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; 8,999,641; 9,840,713, and U.S. Patent Application No. 14/704,551.

Letters of Credit

As of September 30, 2019, the Company had restricted cash of \$3.9 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 September 30, 2019.

Research Agreements

The Company has engaged several research institutions and companies to identify new delivery strategies and applications of its gene-editing technology. The Company is also a party to a number of research license agreements which require significant upfront payments, future royalty payments and potential milestone payments from time to time, as well as intellectual property agreements, which require maintenance and milestone payments from time to time. In association with these agreements, the Company has committed to making payments of \$1.4 million and \$2.3 million in the fourth quarter of 2019 and for the year ended December 31, 2020, respectively. For the three and nine months ended September 30, 2019, the Company paid \$0.2 million and \$2.4 million, respectively, related to these research agreements. For the three and nine months ended September 30, 2018, the Company paid \$0.3 million and \$1.4 million, respectively, related to these research agreements.

The Company is also a party to a number of manufacturing agreements that require upfront payments for the future performance of services. In connection with these agreements, the Company paid \$2.2 million in upfront payments, which were recorded as prepaid expenses as of September 30, 2019. The Company will amortize the prepaid balance as services are performed.

5. Leases

In June 2015, the Company entered into a lease agreement for the lease of research facility space with a commencement date of November 15, 2015 (the "2015 Lease"). The lease expires in February 2022. The 2015 Lease contains escalating rent clauses which require higher rent payments in future years. With the adoption of ASC 842, the Company has recorded a right-of-use asset and corresponding lease liability.

In May 2016, the Company entered into a sublease agreement for its primary office and research facility in Cambridge, Massachusetts, with a commencement date of December 23, 2016 (the "2016 Sublease"). The sublease expires in December 2026, and the Company has an option to extend the term of sublease for an additional five-year period if, at the time of expiration of the initial term, the sublessor does not intend to utilize the space for itself or its affiliates. The 2016 Sublease contains escalating rent clauses which require higher rent payments in future years. With the adoption of ASC 842, the Company has recorded a right-of-use asset and corresponding lease liability. The right-of-use asset and corresponding lease liability does not include the additional five-year period under the option.

In May 2019, the Company entered into a lease agreement for office facility space with a commencement date of June 1, 2019 (the "2019 Lease"). The lease expires in November 2026, and the Company has an option to extend the term of the lease for an additional five-year period based on certain conditions within the Company's control. The 2019 Lease contains escalating rent clauses which require higher rent payments in future years. At lease commencement, the Company recorded a right-of-use asset and corresponding lease liability. The right-of-use asset and corresponding lease liability does not include the additional five-year period under the option.

In addition, the Company rents certain office space in Zug, Switzerland, on a short-term basis for which a right-of-use asset and liability are not recorded, in accordance with the practical expedient elected.

The Company has embedded leases in certain research and license agreements for which the Company has recorded a right of use asset and liability. These arrangements are not significant in comparison to the Company's total operating lease assets and liabilities. In addition, the Company has identified certain short-term leases embedded within its manufacturing contracts which are not recorded on the Company's balance sheet in accordance with the practical expedient elected.

The Company identified and assessed the following estimates in recognizing the right-of-use asset and corresponding liability:

- *Expected lease term:* The expected lease term for those leases commencing prior to January 1, 2019 did not change with the adoption of ASC 842. The expected lease term for leases commencing after the adoption of ASC 842 includes noncancelable lease periods and, when applicable, periods covered by an option to extend the lease if the Company is reasonably certain to exercise that option, as well as periods covered by an option to terminate the lease if the Company is reasonably certain not to exercise that option.
- *Incremental borrowing rate:* As the discount rates in the Company's lease are not implicit, the Company estimated the incremental borrowing rate based on the rate of interest the Company would have to pay to borrow a similar amount on a collateralized basis over a similar term.

The following table summarizes the lease assets and liabilities as of September 30, 2019 (in thousands):

	<u>As of September 30, 2019</u>	
Assets		
Operating lease assets	\$	30,619
Total lease assets		30,619
Liabilities		
Current		
Operating lease liabilities		4,805
Non-current		
Operating lease liabilities, net of current portion		36,827
Total lease liabilities	\$	<u>41,632</u>

The following table summarizes operating lease costs included in research and development and general and administrative expense, as well as sublease income for the three and nine months ended September 30, 2019 (in thousands):

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2019</u>		<u>2019</u>	
Operating lease costs	\$	2,131	\$	5,857
Short-term lease costs		1,117		3,378
Variable lease costs		698		2,111
Sublease income		—		(525)
Net lease cost	\$	<u>3,946</u>	\$	<u>10,821</u>

The following table summarizes the maturity of undiscounted payments due under lease liabilities and the present value of those liabilities as of September 30, 2019 (in thousands):

	Total
2019	\$ 1,997
2020	9,334
2021	8,816
2022	7,345
2023	7,363
Thereafter	23,254
Total	\$ 58,109
Present value adjustment	(16,477)
Present value of lease liabilities	\$ 41,632

The following table summarizes the lease term and discount rate as of September 30, 2019:

	As of September 30, 2019
Weighted-average remaining lease term (years)	
Operating leases	6.8
Weighted-average discount rate	
Operating leases	9.9%

The following table summarizes the cash paid for amounts included in the measurement of lease liabilities for the nine months ended September 30, 2019 (in thousands):

	Nine Months Ended September 30, 2019
Cash paid for amounts included in the measurement of lease liabilities	\$ 6,362
Operating cash flows from operating leases	\$ 6,362

6. 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 (the “2015 Collaboration Agreement”) with Vertex Pharmaceuticals Incorporated and certain of its subsidiaries (“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 (“Amendment No. 1”) and the Joint Development Agreement (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 \$5.0 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 each of these targets, Vertex will lead development and global commercialization activities and the Company has the potential to receive up to \$420.0 million in development, regulatory and commercial milestones and royalties on net product sales for each of the four 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 (the “2019 Collaboration Agreement”) for the development and commercialization of products for the treatment of Duchenne Muscular Dystrophy (“DMD”) and Myotonic Dystrophy Type 1 (“DM1”). Under the terms of the 2019 Collaboration Agreement, the Company received an upfront, nonrefundable payment of \$175 million. In addition, the Company is eligible to receive potential future payments of up to \$825 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 (“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 (“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 right 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 million in potential specified research, development, regulatory and commercial milestones and tiered single-digit royalties on future net sales.

In October 2019, Vertex exercised options granted to it under the 2015 Collaboration Agreement to exclusively license three collaboration targets developed under the 2015 Collaboration Agreement. As a result, the Company is entitled to receive an aggregate of \$30.0 million in option exercise payments from Vertex, which we expect to receive in the fourth quarter.

Accounting for the Vertex Agreements

The 2015 Collaboration Agreement, Amendment No. 1, Amendment No. 2, JDA and 2019 Collaboration Agreement are collectively 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 Vertex Agreements include components of a customer-vendor relationship as defined under ASC 606, *Revenue from Contracts with Customers* (“ASC 606”), collaborative arrangements as defined under ASC 808, *Collaborative Arrangements* (“ASC 808”) and research and development costs as defined under ASC 730, *Research and Development* (“ASC 730”).

Accounting Analysis Under ASC 606

Identification of the Contract(s)

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 (“DMD License”); (ii) an exclusive license for worldwide rights for DM1 gene editing products (“DM1 License”); (iii) the performance of specified guide RNA research for DM1 (“DM1 R&D Services”); (iv) a material right representing the option to obtain a co-exclusive development and commercialization license for a specified target (“Specified Target Option”); (v) three material rights representing the option for up to three exclusive licenses to develop and commercialize the collaboration targets (“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 represents 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; 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 September 30, 2019. 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 (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 \$24.6 million and \$76.2 million, respectively. The ESSP was determined based on probability and present value adjusted cash flows from projected worldwide net profit or net loss 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.

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 or net loss 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.

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.

The aforementioned ESSP’s reflect the level of risk and expected probability of success inherent in the nature associated 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 \$0.0 million, or the contractual value of the option.

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.

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 Company concluded that the Specified Target Option and Collaboration Target Options are considered material rights under the Vertex Agreements. As a result, revenue related to these material rights will be recognized at a point in time, upon the exercise of the option by Vertex or expiration.

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 will recognize 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.

The Company recognized \$211.9 million and \$212.1 million of revenue related to the Vertex Agreements for the three and nine months ended September 30, 2019, respectively. The Company recognized \$0.1 million and \$0.5 million of revenue related to the Vertex Agreements for the three and nine months ended September 30, 2018. As of September 30, 2019, there was \$47.7 million of current deferred revenue and \$11.9 million non-current deferred revenue related to the Vertex Agreements. The amounts classified as current deferred revenue primarily relate to the Collaboration Target Options which expire less than one year from September 30, 2019. The amounts classified as non-current deferred revenue primarily relate to the Specified Target Option, which is not expected to occur within one year from September 30, 2019. The transaction price allocated to the remaining performance obligations was \$59.6 million.

Milestones under the Vertex Agreements

The Company has evaluated the milestones that may be received in connection with the Vertex Agreements. In connection with the Collaboration Target Options, the Company is eligible to receive up to \$420.0 million (inclusive of \$10 million due upon exercise of each exclusive option) in development, regulatory and commercial milestones and royalties on net product sales for each of the three collaboration targets. 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.

In connection with the JDA, the Company 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. Revenue was recognized for this milestone in the third quarter of 2019, the point in time in which the milestone was both probable and achieved.

The Company is eligible to receive potential future payments of up to \$825 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. This amount is recorded as a contract asset within prepaid expenses and other current assets 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.

With the exception of the first research milestone of \$25.0 million, each of the remaining milestones are fully constrained as of September 30, 2019. 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.

As discussed above, in October 2019, Vertex exercised its three Collaboration Target Options. As a result, the Company will record revenue of \$76.7 million during the fourth quarter of 2019, inclusive of \$30 million in cash received related to the option exercises, as well as \$46.7 million of previously deferred revenue allocated to the three Collaboration Target Options.

Accounting Analysis under ASC 808

The Company did not identify any additional collaborative elements as a result of the 2019 Agreements. As such, the following collaborative elements which are accounted for under ASC 808 remain in place: (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.

The Company recognized \$7.5 million and \$21.3 million of research and development expense related to the Vertex Agreements for the three and nine months ended September 30, 2019, respectively. The Company recognized \$8.8 million and \$25.4 million of research and development expense related to the collaboration with Vertex for the three and nine months ended September 30, 2018, respectively. Research and development expense for the three and nine months ended September 30, 2019 was net of \$3.8 million and \$11.8 million of reimbursements from Vertex, respectively. Research and development expense for the three and nine months ended September 30, 2018 was net of \$3.7 million and \$10.4 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 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, it 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 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

On December 19, 2015, the Company entered into an agreement with Bayer Healthcare LLC and certain of its affiliates (“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 Therapeutics LLP (“Casebia”). Bayer and the Company each received a 50% equity interest in the entity in exchange for their respective contributions to the entity. The Company contributed \$0.1 million in cash and licensed its proprietary CRISPR/Cas9 gene editing technology and intellectual property for selected disease indications. Bayer contributed its protein engineering expertise and relevant disease know-how. Under the agreement, Casebia paid the Company \$35.0 million in exchange for a worldwide, exclusive license to commercialize the Company’s gene-editing technology specifically for the indications covered by the license. There are no milestone, royalties or other payments due to the Company under this aspect of the agreement. The Company also entered into a separate services agreement with Casebia, under which the Company agreed to provide compensated research and development services.

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 has no further contractual obligations to provide cash financing to Casebia and accordingly, no additional losses have been recorded beyond the initial equity amount. Casebia’s net losses were \$22.6 million and \$54.9 million for the three and nine months ended September 30, 2019, respectively. Casebia’s net losses were \$13.5 million and \$25.8 million for the three and nine months ended September 30, 2018, respectively. Unrecognized equity method losses in excess of the Company’s equity investment in Casebia were \$0.1 million and \$45.3 million as of September 30, 2019 and December 31, 2018, respectively.

The remaining performance obligations include research and development services, which are recorded as revenue under ASC 606 and cost sharing activities with Casebia related to shared research and technology licenses are accounted for as a cost/profit sharing arrangement under ASC 808, with the related impact of the cost sharing included as research and development expense. During the three and nine months ended September 30, 2019, the Company recognized \$0.1 million and \$0.5 million of revenue, respectively, related to the collaboration with Casebia. During the three and nine months ended September 30, 2018, the Company recognized \$0.9 million and \$2.0 million of revenue, respectively, related to the collaboration with Casebia. During the three and nine months ended September 30, 2019, the Company recognized \$0.1 million and \$0.7 million, respectively, of research and development expense related to the collaboration with Casebia. During the three and nine months ended September 30, 2018, the Company recognized \$1.2 million and \$2.4 million, respectively, of research and development expense related to the collaboration with Casebia. During the three and nine months ended September 30, 2019, the Company recognized a loss from equity method investment of \$3.3 million and \$5.5 million, respectively, related to stock-based compensation expense for Casebia employees. During the three and nine months ended September 30, 2018, the Company recognized a loss from equity method investment of \$1.2 million and \$2.2 million, respectively, related to stock-based compensation expense for Casebia employees.

7. Share Capital

The Company had 55,677,088 authorized common shares as of September 30, 2019, with a par value of CHF0.03 per share. Included in the authorized common shares as of September 30, 2019 are 256,989 treasury shares which are legally outstanding but not considered outstanding for accounting purposes and 230,729 shares registered and reserved for future issuance. The Company had conditional capital reserved for future issuance of 19,028,428 common shares for employee benefit plans and 4,919,700 common shares for debt instruments as of September 30, 2019. Under Swiss law, authorized share capital consisted of 25,134,003 common shares as of September 30, 2019.

At-the-Market Offering

In August 2018, the Company entered into an Open Market Sale AgreementSM (the “2018 ATM”) with Jefferies LLC (“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 the first quarter of 2019, the Company began to issue and sell securities under this sales agreement. During the three and nine months ended September 30, 2019, the Company sold 1,452,880 and 2,816,568 common shares, respectively, for net cash proceeds of \$69.4 million and \$121.9 million, respectively, after deducting commission fees of \$1.5 million and \$3.1 million, respectively. In addition, the Company paid approximately \$0.5 million and \$0.7 million in stamp taxes during the three and nine months ended September 30, 2019, respectively, and accrued an additional \$0.7 million for stamp taxes as of September 30, 2019 related to securities sold under the 2018 ATM.

In August 2019, following the termination of the 2018 ATM by its terms, the Company entered into a new Open Market Sale AgreementSM with Jefferies (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. The Company has not yet issued or sold any securities under the 2019 ATM.

8. Stock-based Compensation

During the three and nine months ended September 30, 2019 and 2018, the Company recognized the following stock-based compensation expense (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Research and development	\$ 6,262	\$ 3,999	\$ 17,137	\$ 12,081
General and administrative	5,081	4,056	15,063	9,879
Loss from equity method investment	3,430	1,012	5,467	3,256
Total	<u>\$ 14,773</u>	<u>\$ 9,067</u>	<u>\$ 37,667</u>	<u>\$ 25,216</u>

Stock option activity

The following table summarizes stock option activity for the nine months ended September 30, 2019 (intrinsic value in thousands):

	Stock Options	Weighted- Average Exercise Price	Weighted- Average Remaining Contractual Term (years)	Aggregate Intrinsic Value
Outstanding at December 31, 2018	6,689,311	\$ 25.42	8.3	\$ 68,572
Granted	2,136,873	\$ 37.23		
Exercised	(475,062)	\$ 12.03		
Cancelled or forfeited	(337,020)	\$ 31.50		
Outstanding at September 30, 2019	8,014,102	\$ 29.10	8.0	\$ 119,011
Exercisable at September 30, 2019	3,544,643	\$ 21.65	7.1	\$ 76,945
Vested and expected to vest at September 30, 2019	8,014,102	\$ 29.10	8.0	\$ 119,011

The Company estimated the fair value of each stock option award using the Black-Scholes option-pricing model based on the following assumptions:

Assumptions	Nine Months Ended September 30,	
	2019	2018
Weighted-average expected volatility	69.2%	71.9%
Expected term (in years)	6.0	6.0
Risk-free interest rate	2.4%	2.7%
Expected dividend yield	0.0%	0.0%

As of September 30, 2019, total unrecognized compensation expense related to stock options was \$9.3 million which the Company expects to recognize over a remaining weighted-average period of 2.8 years.

In May 2018, the Company modified the terms of certain options held by a departing employee. The modification resulted in \$2.2 million in stock-based compensation expense recorded during the period. In September 2019, the Company modified the terms of certain options held by non-employees. The modifications resulted in \$2.9 million in stock-based compensation expense recorded during the period.

Restricted stock activity

The following table summarizes restricted stock activity for the nine months ended September 30, 2019:

	Restricted Stock	Weighted- Average Grant Date Fair Value
Unvested balance as of December 31, 2018	327,342	\$ 36.72
Granted	145,000	43.61
Vested	(69,091)	15.07
Cancelled or forfeited	(23,000)	41.64
Unvested balance as of September 30, 2019	380,251	\$ 42.98

As of September 30, 2019, total unrecognized compensation expense related to unvested restricted common shares was \$0.5 million which the Company expects to recognize over a remaining weighted-average vesting period of 1.42 years.

9. Net Income (Loss) Per Share Attributable to Common Shareholders

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

The following table sets forth the computation of basic and diluted net income (loss) per share for the periods ended (in thousands, except per share amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
<i>Basic net income (loss) per common share calculation:</i>				
Net income (loss) attributable to common shareholders	\$ 138,423	\$ (50,711)	\$ 36,316	\$ (117,391)
Net income (loss) attributable to common shareholders - basic	\$ 138,423	\$ (50,711)	\$ 36,316	\$ (117,391)
Basic weighted-average common shares outstanding	54,829,057	47,391,988	53,380,123	46,709,388
Basic net income (loss) per common share	\$ 2.52	\$ (1.07)	\$ 0.68	\$ (2.51)
<i>Diluted net income (loss) per common share calculation:</i>				
Net income (loss) attributable to common shareholders	\$ 138,423	\$ (50,711)	\$ 36,316	\$ (117,391)
Net income (loss) attributable to common shareholders - diluted	\$ 138,423	\$ (50,711)	\$ 36,316	\$ (117,391)
Weighted-average shares used to compute basic net income (loss) per common share	54,829,057	47,391,988	53,380,123	46,709,388
<i>Effect of potentially dilutive securities:</i>				
Outstanding options	2,612,354	—	2,326,824	—
Unvested restricted common shares	157,490	—	114,473	—
Weighted-average shares used to compute diluted net income (loss) per common share	57,598,901	47,391,988	55,821,420	46,709,388
Diluted net income (loss) per common share	\$ 2.40	\$ (1.07)	\$ 0.65	\$ (2.51)

The Company did not include the securities in the following table in the computation of the net income (loss) per share calculations because the effect would have been anti-dilutive during each period:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Outstanding options	2,612,354	6,698,579	2,326,824	6,698,579
Unvested restricted common shares	157,490	178,884	114,473	178,884
Total	2,769,844	6,877,463	2,441,297	6,877,463

10. Income Taxes

During the three and nine months ended September 30, 2019, the Company recorded an income tax provision of \$0.3 million and \$0.4 million, respectively, representing an effective tax rate of 0.2% and 1.2%, respectively. During the three and nine months ended September 30, 2018, the Company recorded an income tax provision of \$0.1 million and \$0.3 million, respectively, representing an effective tax rate of 0.3% and -0.3%, 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.

11. Related Party Transactions

In the fourth quarter of 2018, upon becoming an owner of record of more than 10% of the voting interest of the Company, Vertex became a related party under ASC 850, Related party disclosures. Refer to Note 6 for discussion of transactions with Casebia and Vertex, related parties.

The Company is a party to intellectual property license agreements with Dr. Emmanuelle Charpentier. For the three and nine months ended September 30, 2019, the Company paid Dr. Charpentier approximately \$1.2 million of sublicense fees in research and development expense primarily related to the Vertex Agreements.

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, 2018 filed with the Securities and Exchange Commission, or the SEC, on February 25, 2019. 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, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2018, our actual results or timing of certain events could differ materially from the results or timing described in, or implied by, these forward-looking statements.

Overview

We are a leading gene editing company focused on the development of CRISPR/Cas9-based therapeutics. CRISPR/Cas9 is a revolutionary gene editing technology that allows for precise, directed changes to genomic DNA. The application of CRISPR/Cas9 for gene editing was co-invented by one of our scientific founders, Dr. Emmanuelle Charpentier, 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.

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.

We have established a portfolio of therapeutic programs across a broad range of disease areas including hemoglobinopathies, oncology, regenerative medicine and rare diseases. We are enrolling clinical trials in the United States, Europe and Canada for CTX001, which is an investigational, autologous, gene-edited hematopoietic stem cell therapy for the treatment of transfusion-dependent beta thalassemia ("TDT") and severe sickle cell disease ("SCD"). Additionally, we are developing our own portfolio of CAR-T cell product candidates based on our gene-editing technology and have begun treating patients in a Phase 1/2 trial to assess the safety and efficacy of CTX110, our wholly-owned allogeneic CAR-T cell therapy targeting CD19+ malignancies. The multi-center, open label trial is designed to enroll up to 95 patients and investigate several dose levels of CTX110. The trial is currently enrolling at clinical trial sites in the United States and Australia. In addition, the Company obtained approval from Health Canada for its Clinical Trial Application.

All of our revenue to date has been collaboration revenue. We have incurred significant net operating losses in every year since our inception and expect to continue to incur net operating losses for the foreseeable future. As of September 30, 2019, we had \$629.7 million in cash and cash equivalents and an accumulated deficit of \$255.3 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.

Collaborations

We entered into a series of strategic research agreements with Vertex Pharmaceuticals Incorporated and certain of its subsidiaries ("Vertex") in October 2015, December 2017 and June 2019. Under the 2015 and 2017 agreements with Vertex we are focused on developing CRISPR/Cas9-based therapies, including the development and commercialization of CTX001 for TDT and severe SCD.

As part of our collaboration with Vertex, we are currently investigating CTX001 in a Phase 1/2 open-label clinical trial 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 and in a second Phase 1/2 open-label clinical trial designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 18 to 35 with severe SCD. The first two patients in each trial will be treated sequentially and, pending data from the initial two patients in each trial, each trial will open for broader concurrent enrollment, respectively.

Enrollment is ongoing at clinical trial sites in the United States, Canada and Europe for the Phase 1/2 trial of CTX001 in patients with TDT and at clinical trial sites in the United States, Canada and Europe for the trial in patients with severe SCD. In addition, CTX001 has been granted Fast Track Designation by the U.S. Food and Drug Administration for the treatment of TDT and severe SCD, and, recently, the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion for orphan drug designation (ODD) of CTX001 for the treatment of TDT. In addition, we are expanding the TDT patient population for CTX001 to include beta zero/beta zero subtypes.

In June 2019, we entered into a strategic development and collaboration agreement with Vertex, or the 2019 Collaboration Agreement, for the development and commercialization of products for the treatment of Duchenne Muscular Dystrophy and Myotonic Dystrophy Type 1 (“DM1”). The 2019 Collaboration Agreement was not effective until after regulatory review, which occurred in July 2019. Under the terms of the 2019 Collaboration Agreement, we received an upfront, nonrefundable payment of \$175.0 million. Additionally, under the terms of the 2019 Collaboration Agreement, we have an option, exercisable during a specified exercise period, to co-develop and co-commercialize products for the treatment of DM1.

In October 2019, Vertex exercised the options granted under the collaboration it established with us in 2015 to in-license three additional targets for the development of gene-based treatments using CRISPR-based gene editing. The targets include the cystic fibrosis transmembrane conductance regulator (CFTR) gene and two undisclosed targets. Under the terms of the agreement, we will receive an upfront payment of \$30 million in connection with the option exercise and have the potential to receive up to \$410 million in development, regulatory and commercial milestones and royalties on net product sales for each of the three targets, and Vertex will receive exclusive rights to develop and commercialize products related to these targets globally. The research term of our 2015 collaboration with Vertex has now expired, and Vertex no longer holds rights to in-license additional targets under that agreement.

We also entered into an agreement (the “JV Agreement”) with Bayer HealthCare LLC (“Bayer”) in December 2015 to create a joint venture, Casebia Therapeutics LLP (“Casebia” or the “JV”) 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. We and Bayer each have a 50% interest in the JV. Under the JV Agreement, Bayer is making available its protein engineering expertise and relevant disease know-how and we are contributing our proprietary CRISPR/Cas9 gene editing technology and intellectual property. Bayer will also provide up to \$300.0 million in research and development investments to the JV over the first five years, subject to specified conditions. In October 2019, we and Bayer announced proposed plans whereby Casebia would operate under our direct management. Upon closing of the transaction, Casebia would focus on the development of its lead programs in hemophilia, ophthalmology and autoimmune diseases, with Bayer having opt-in rights for two products at Investigational New Drug (“IND”) submission. We and Bayer are negotiating the definitive agreements and, subject to the finalization of the definitive agreement and satisfaction of closing conditions, anticipate closing the transaction in the fourth quarter of 2019.

Refer to Note 6 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.

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. During the three and nine months ended September 30, 2019, we recognized \$211.9 million and \$212.6 million of revenue related to our collaboration arrangements with Vertex and Casebia, respectively. During the three and nine months ended September 30, 2018, we recognized \$0.6 million and \$3.0 million of revenue related to our collaboration agreements with Vertex and Casebia, respectively. As of September 30, 2019, we received one upfront, nonrefundable payment of \$175.0 million, recorded a \$25.0 million contract asset, and received a one-time low single-digit milestone payment under the Vertex collaboration agreements. 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, 2018 filed with the SEC on February 25, 2019, as well as Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

Research and Development Expenses

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

- employee-related expenses, including salaries, benefits and equity-based compensation expense;
- costs of services performed by third parties that conduct research and development and preclinical 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.

Research and development costs are expensed as incurred. Nonrefundable advance payments for research and development goods or services to be received in the future are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed. At this time, we cannot reasonably estimate or know the nature, timing or estimated costs of the efforts that will be necessary to complete the development of any product candidates we may identify and develop. This is due to the numerous risks and uncertainties associated with developing such product candidates, including the uncertainty of:

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

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

Except for activities we perform in connection with our collaborations with Vertex and Casebia, 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 research and development costs to increase significantly for the foreseeable future as our current development programs progress and new programs are added.

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. 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 September 30, 2019 and 2018 (in thousands):

	Three Months Ended September 30,		Period to Period
	2019	2018	Change
Collaboration revenue	\$ 211,928	\$ 563	\$ 211,365
Operating expenses:			
Research and development	57,246	\$ 39,820	17,426
General and administrative	15,519	\$ 10,175	5,344
Total operating expenses	72,765	49,995	22,770
Income (loss) from operations	139,163	(49,432)	188,595
Other expense, net	(466)	\$ (1,142)	676
Net income (loss) before income taxes	138,697	(50,574)	189,271
Provision for income taxes	(274)	\$ (137)	(137)
Net income (loss)	\$ 138,423	\$ (50,711)	\$ 189,134

Collaboration Revenue

Collaboration revenue for the three months ended September 30, 2019 was \$211.9 million, compared to \$0.6 million for the three months ended September 30, 2018. The increase of approximately \$211.4 million was primarily attributable to revenue recognized in connection with our collaboration with Vertex. Please refer to Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for further information.

Research and Development Expenses

Research and development expenses were \$57.2 million for the three months ended September 30, 2019, compared to \$39.8 million for the three months ended September 30, 2018. The increase of approximately \$17.4 million was primarily attributable to the following:

- \$7.8 million of increased employee compensation, benefit and other headcount related expenses, of which \$2.3 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth;
- \$9.0 million of increased variable research and development costs;
- \$3.5 million of increased facility-related expenses; offset by,
- \$3.6 million of decreased license fees, primarily attributable to non-cash consideration of \$15.0 million expensed related to the ViaCyte, Inc. collaboration in the third quarter of 2018, compared to \$10.0 million of non-cash consideration expensed related to our collaboration with Vertex in the third quarter of 2019.

General and Administrative Expenses

General and administrative expenses were \$15.5 million for the three months ended September 30, 2019, compared to \$10.2 million for the three months ended September 30, 2018. The increase of approximately \$5.3 million was primarily attributable to \$2.4 million of increased employee compensation, benefit and other headcount related expenses, of which \$1.0 million is stock-based compensation expense, primarily due to an increase in headcount to support overall growth.

Other Expense, Net

Other expense was \$0.5 million for the three months ended September 30, 2019, compared to \$1.1 million of expense for the three months ended September 30, 2018. The change was primarily due to the loss from equity method investment, offset by interest income earned on cash and cash equivalents for the three months ended September 30, 2019.

Comparison of nine months ended September 30, 2019 and 2018 (in thousands):

	Nine Months Ended September 30,		Period to Period Change
	2019	2018	
Collaboration revenue	\$ 212,574	\$ 3,009	\$ 209,565
Operating expenses:			
Research and development	130,601	\$ 84,972	45,629
General and administrative	46,216	\$ 31,752	14,464
Total operating expenses	<u>176,817</u>	<u>116,724</u>	<u>60,093</u>
Income (loss) from operations	35,757	(113,715)	149,472
Other income (expense), net	1,003	(3,357)	4,360
Net income (loss) before income taxes	36,760	(117,072)	153,832
Provision for income taxes	(444)	(319)	(125)
Net income (loss)	<u>\$ 36,316</u>	<u>\$ (117,391)</u>	<u>\$ 153,707</u>

Collaboration Revenue

Collaboration revenue for the nine months ended September 30, 2019 was \$212.6 million, compared to \$3.0 million for the nine months ended September 30, 2018. The increase of approximately \$209.6 million was primarily attributable to revenue recognized in connection with our collaboration with Vertex. Please refer to Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for further information.

Research and Development Expenses

Research and development expenses were \$130.6 million for the nine months ended September 30, 2019, compared to \$85.0 million for the nine months ended September 30, 2018. The increase of approximately \$45.6 million was primarily attributable to the following:

- \$17.9 million of increased employee compensation, benefit, and other headcount related expenses, of which \$5.1 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth;
- \$18.2 million of increased variable research and development costs;
- \$2.5 million of increased professional and consulting fees;
- \$8.4 million of increased facility-related expenses; offset by,
- \$2.7 million of decreased license fees, primarily attributable to non-cash consideration of \$15.0 million expensed related to the Viacyte, Inc. collaboration in the third quarter of 2018, compared to \$10.0 million of non-cash consideration expensed related to our collaboration with Vertex in the third quarter of 2019.

General and Administrative Expenses

General and administrative expenses were \$46.2 million for the nine months ended September 30, 2019, compared to \$31.8 million for the nine months ended September 30, 2018. The increase of approximately \$14.5 million was primarily attributable to the following:

- \$8.0 million of increased employee compensation, benefit, and other headcount related expenses, of which \$5.2 million is stock-based compensation expense, primarily due to an increase in headcount to support overall growth;
- \$2.2 million of increased legal, professional and consulting fees;
- \$1.7 million of increased intellectual property costs; and,
- \$1.0 million of increased facility-related expenses.

Other Income (Expense), Net

Other income was \$1.0 million for the nine months ended September 30, 2019, compared to \$3.4 million of expense for the nine months ended September 30, 2018. The change was primarily due to interest income earned on cash and cash equivalents for the nine months ended September 30, 2019, partially offset by the loss from equity method investments.

Liquidity and Capital Resources

As of September 30, 2019, we had cash and cash equivalents of approximately \$629.7 million of which approximately \$622.8 million was held outside of the United States. In August 2018, we entered into an Open Market Sale AgreementSM (the “2018 ATM”) with Jefferies LLC (“Jefferies”), under which Jefferies was able to sell, from time to time, common shares having aggregate gross proceeds of up to \$125.0 million. In the first quarter of 2019, we began to issue and sell securities under this sales agreement. During the three and nine months ended September 30, 2019, we sold 1,452,880 and 2,816,568 common shares, respectively, for net cash proceeds of \$69.4 million and \$121.9 million, respectively, after deducting commission fees of \$1.5 million and \$3.1 million, respectively. In addition, we paid approximately \$0.5 million and \$0.7 million in stamp taxes during the three and nine months ended September 30, 2019, respectively, and accrued an additional \$0.7 million for stamp taxes as of September 30, 2019 related to securities sold under the 2018 ATM. In August 2019, following the termination of our 2018 ATM by its terms, we entered into a new Open Market Sale AgreementSM with Jefferies (the “2019 ATM”), under which we may offer and sell, from time to time, common shares having aggregate gross proceeds up to \$200.0 million. We have not yet issued or sold any securities under the 2019 ATM.

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 the current JV Agreement with Bayer for Casebia and our collaborations with Vertex. Except for these sources 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. 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 collaborations with Vertex and Bayer 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):

	Nine Months Ended September 30,		Period to Period
	2019	2018	Change
Net cash provided by (used in) operating activities	\$ 53,301	\$ (67,867)	\$ 121,168
Net cash used in investing activities	(5,732)	(1,773)	(3,959)
Net cash provided by financing activities	126,265	317,203	(190,938)
Effect of exchange rate changes on cash	(14)	(15)	1
Net increase (decrease) in cash	<u>\$ 173,820</u>	<u>\$ 247,548</u>	<u>\$ (73,728)</u>

Net Cash Provided by (Used in) Operating Activities

Net cash provided by operating activities was \$53.3 million for the nine months ended September 30, 2019, compared to cash used of \$67.9 million for the nine months ended September 30, 2018. The \$121.1 million increase in cash provided by operating activities was driven by the increase in net income during this period of \$153.7 million due to our collaboration with Vertex. The increase was offset by an increase in prepaid and other current assets as a result of recording a \$25.0 million contract asset related to our collaboration with Vertex as of September 30, 2019.

Net Cash Used in Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2019 was \$5.7 million, compared to \$1.8 million for the nine months ended September 30, 2018. 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 nine months ended September 30, 2019 was \$126.3 million, compared with \$317.2 million for the nine months ended September 30, 2018. The net cash provided by financing activities for the nine months ended September 30, 2019 consisted of proceeds from the issuance of common shares in connection with the 2018 ATM, which resulted in \$121.9 million of net cash proceeds, after deducting \$3.1 million in commissions and \$0.7 million in stamp taxes, as well as the exercise of stock options. The net cash provided by financing activities for the nine months ended September 30, 2018 consisted of proceeds from the issuance of common shares in an offering in January of 2018 which resulted in \$122.6 million of net proceeds to the Company, proceeds from the issuance of common shares in an offering in September of 2018 which resulted in \$187.6 million of net proceeds to the Company, as well as exercises of stock options. In addition, \$3.1 million of stamp taxes on the issuance proceeds from the January and September offerings were recorded as an offset to additional paid in capital.

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, 2018 filed with the SEC on February 25, 2019. There have been no material changes from the contractual commitments and obligations previously disclosed in our Annual Report on Form 10-K other than the changes described in Note 5 and Note 6 of the notes to our unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q.

Off-Balance Sheet Arrangements

As of September 30, 2019, we do 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, 2018 filed with the SEC on February 25, 2019.

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

Foreign Exchange Market 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 September 30, 2019, 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 September 30, 2019, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

During the quarter ended September 30, 2019, there were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

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

As disclosed in our Current Report on Form 8-K filed with the SEC on June 26, 2019, on June 25, 2019, we received notification that the United States Patent and Trademark Office initiated an interference proceeding at the Patent Trial and Appeal Board (the “PTAB”) between certain pending U.S. patent applications co-owned by the University of California, the University of Vienna and Dr. Emmanuelle Charpentier (collectively, the “CVC Group”) and certain patents and a patent application currently owned by the Broad Institute and Massachusetts Institute of Technology and, in some instances, the President and Fellows of Harvard College (individually and collectively, the “Broad”), all of which are related to the single guide format of CRISPR/Cas9 genome editing technology in eukaryotic cells. We have an exclusive worldwide license in the field of human therapeutics to Dr. Charpentier’s rights as a co-owner of the CVC Group portfolio. Specifically, the PTAB has declared Patent Interference No. 106,115 between the CVC Group’s pending U.S. Patent Application Nos. 15/947,680; 15/947,700; 15/947,718; 15/981,807; 15/981,808; 15/981,809; 16/136,159; 16/136,165; 16/136,168; and 16/136,175, and the Broad’s U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; 8,999,641; 9,840,713, and U.S. Patent Application No. 14/704,551.

Item 5. Other Information

In October 2019, we and Bayer announced proposed plans whereby Casebia would operate under our direct management. Upon closing of the transaction, Casebia would focus on the development of its lead programs in hemophilia, ophthalmology and autoimmune diseases, with Bayer having opt-in rights for two products at IND submission. The transaction is subject to negotiation and execution of definitive agreements as well as certain customary conditions. We and Bayer are negotiating the definitive agreements and, subject to the finalization of the definitive agreement and satisfaction of closing conditions, we anticipate closing the transaction in the fourth quarter of 2019.

In October 2019, Vertex exercised the options granted under the collaboration it established with us in 2015 to in-license three additional targets for the development of gene-based treatments using CRISPR-based gene editing. The targets include the cystic fibrosis transmembrane conductance regulator (CFTR) gene and two undisclosed targets. Under the terms of the agreement, we will receive an upfront payment of \$30 million in connection with the option exercise and have the potential to receive up to \$410 million in development, regulatory and commercial milestones and royalties on net product sales for each of the three targets, and Vertex will receive exclusive rights to develop and commercialize products related to these targets globally. The research term of our 2015 collaboration with Vertex has now expired, and Vertex no longer holds rights to in-license additional targets under that agreement.

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
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase
104*	Cover Page Interactive Data File – The cover page interactive data file does not appear in the interactive data file because its XBRL tags are embedded within the inline XBRL document.

* 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: October 28, 2019

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

Dated: October 28, 2019

By: /s/ Michael Tomsicek
Michael Tomsicek
Chief Financial Officer
(Principal Financial Officer)

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: October 28, 2019

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

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: October 28, 2019

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

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

In connection with the Quarterly Report on Form 10-Q of CRISPR Therapeutics AG (the "Company") for the period ended September 30, 2019 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)

October 28, 2019

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

October 28, 2019

