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 June 30, 2021

OR

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

For the transition period from _____ to _____.

Commission file number: 001-37923

CRISPR THERAPEUTICS AG

(Exact name of Registrant as specified in its charter)

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

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

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

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Shares, nominal value CHF 0.03	**CRSP**	**The Nasdaq Global Market**

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

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

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

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

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

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

As of July 27, 2021, there were 76,201,185 shares of registrant's common shares outstanding.

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

"CRISPR Therapeutics®" standard character mark and design logo, "CTX001™," "CTX110™," "CTX120™," and "CTX130™" are trademarks and registered trademarks of CRISPR Therapeutics AG. All other trademarks and registered trademarks contained in this Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, trademarks, service marks and trade names referred to in this Quarterly Report on Form 10-Q may appear without the ® or ™ symbols and any such omission is not intended to indicate waiver of any such rights.

FORWARD-LOOKING STATEMENTS

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

- *the safety, efficacy and clinical progress of our various clinical programs including those for CTX001™, CTX110™, CTX120™ and CTX130™;*
- *the status of clinical trials, development timelines and discussions with regulatory authorities related to product candidates under development by us and our collaborators;*
- *the initiation, timing, progress and results of our preclinical studies and clinical trials, including our ongoing clinical trials and any planned clinical trials for CTX001, CTX110, CTX120 and CTX130, and our research and development programs, including delays or disruptions in clinical trials, non-clinical experiments and investigational new drug application-enabling studies;*
- *the actual or potential benefits of U.S. Food and Drug Administration, or FDA, designations, such as orphan drug, fast track and regenerative medicine advanced therapy, or such European equivalents, including Priority Medicines (PRIME) designation;*
- *our ability to advance product candidates into, and successfully complete, clinical trials;*
- *our plan to consolidate our U.S. offices in the greater Boston area into a single location and to build-out a cell-therapy manufacturing facility;*
- *our intellectual property coverage and positions, including those of our licensors and third parties as well as the status and potential outcome of proceedings involving any such intellectual property;*
- *our anticipated expenses, ability to obtain funding for our operations and the sufficiency of our cash resources;*
- *the therapeutic value, development, and commercial potential of CRISPR/Cas9 gene-editing technologies and therapies; and*
- *potential impacts due to the coronavirus pandemic such as delays, interruptions or other adverse effects to clinical trials, delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy, and the overall impact of the coronavirus pandemic on our business, financial condition and results of operations.*

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and assumptions that could cause our actual results and the timing of certain events to differ materially from future results expressed or implied by the forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified herein, and those discussed in the section titled "Risk Factors," set forth in Part II, Item 1A of this Quarterly Report on Form 10-Q, if any, our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on February 16, 2021, and in other SEC filings. You should not rely upon forward-looking statements as predictions of future events. Such forward-looking statements speak only as of the date of this report. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make or enter into.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results, performance or achievements may be materially different from what we expect. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

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

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements

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

	As of	
	June 30, 2021	**December 31, 2020**
Assets		
Current assets:		
Cash and cash equivalents	$ 1,646,646	$ 1,168,620
Marketable securities	942,800	521,713
Accounts receivable	150	144
Prepaid expenses and other current assets	31,347	26,143
Total current assets	2,620,943	1,716,620
Property and equipment, net	75,414	42,160
Intangible assets, net	153	180
Restricted cash	18,071	16,848
Operating lease assets	179,381	50,865
Other non-current assets	5,557	1,293
Total assets	$ 2,899,519	$ 1,827,966
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$ 10,145	$ 9,094
Accrued expenses	67,101	53,782
Deferred revenue, current	2,071	2,341
Accrued tax liabilities	4,463	10,473
Operating lease liabilities	15,875	11,362
Other current liabilities	268	7,207
Total current liabilities	99,923	94,259
Deferred revenue, non-current	11,775	11,776
Operating lease liabilities, net of current portion	175,329	50,067
Other non-current liabilities	8,697	7,630
Total liabilities	295,724	163,732
Commitments and contingencies, see Note 6		
Shareholders' equity:		
Common shares, CHF 0.03 par value, 145,364,335 and 115,172,786 shares authorized at June 30, 2021 and December 31, 2020, respectively, 76,273,769 and 74,110,160 shares issued at June 30, 2021 and December 31, 2020, respectively, 76,078,453 and 73,914,844 shares outstanding at June 30, 2021 and December 31, 2020, respectively.	2,352	2,277
Treasury shares, at cost, 195,316 shares at June 30, 2021 and December 31, 2020.	(63)	(63)
Additional paid-in capital	2,529,649	2,235,679
Retained earnings (deficit)	72,486	(573,576)
Accumulated other comprehensive loss	(629)	(83)
Total shareholders' equity	2,603,795	1,664,234
Total liabilities and shareholders' equity	$ 2,899,519	$ 1,827,966

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 June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Revenue:				
Collaboration revenue	$ 900,202	$ 44	$ 900,404	$ 201
Grant revenue	499	—	836	—
Total revenue	900,701	44	901,240	201
Operating expenses:				
Research and development	108,277	59,380	198,842	113,573
General and administrative	29,806	21,353	54,323	40,903
Total operating expenses	138,083	80,733	253,165	154,476
Income (loss) from operations	762,618	(80,689)	648,075	(154,275)
Other income:				
Other income, net	750	1,412	2,705	5,644
Total other income, net	750	1,412	2,705	5,644
Net income (loss) before income taxes	763,368	(79,277)	650,780	(148,631)
Provision for income taxes	(4,143)	(379)	(4,718)	(756)
Net income (loss)	759,225	(79,656)	646,062	(149,387)
Foreign currency translation adjustment	5	(3)	10	(28)
Unrealized loss on marketable securities	(173)	—	(556)	—
Comprehensive income (loss)	$ 759,057	$ (79,659)	$ 645,516	$ (149,415)
Net income (loss) per common share — basic	$ 10.01	$ (1.30)	$ 8.57	$ (2.44)
Basic weighted-average common shares outstanding	75,826,594	61,420,746	75,418,160	61,134,214
Net income (loss) per common share — diluted	$ 9.44	$ (1.30)	$ 8.03	$ (2.44)
Diluted weighted-average common shares outstanding	80,449,956	61,420,746	80,458,855	61,134,214

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

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

	Common Shares		Treasury Shares					
	Shares	CHF 0.03 Par Value	Shares	Amount, at cost	Additional Paid-in Capital	Retained Earnings (Deficit)	Accumulated Other Comprehensive Income (Loss)	Total Shareholders' Equity
Balance at December 31, 2019	60,783,799	1,847	250,226	(63)	1,162,345	(224,711)	7	939,425
Vesting of restricted shares	5,000	—	—	—	—	—	—	-
Exercise of vested options	83,406	3	—	—	1,385	—	—	1,388
Stock-based compensation expense	—	—	—	—	14,151	—	—	14,151
Issuance of common shares related to license agreement	17,830	—	(17,830)	—	889	—	—	889
Other comprehensive loss	—	—	—	—	—	—	(25)	(25)
Net loss	—	—	—	—	—	(69,731)	—	(69,731)
Balance at March 31, 2020	60,890,035	$ 1,850	232,396	$ (63)	$ 1,178,770	$ (294,442)	$ (18)	$ 886,097
Issuance of common shares, net of issuance costs of $3.1 million	1,238,453	38	—	—	82,151	—	—	82,189
Vesting of restricted shares	29,916	1	—	—	—	—	—	1
Exercise of vested options	394,101	11	(37,080)	—	6,334	—	—	6,345
Stock-based compensation expense	—	—	—	—	15,697	—	—	15,697
Other comprehensive loss	—	—	—	—	—	—	(3)	(3)
Net loss	—	—	—	—	—	(79,656)	—	(79,656)
Balance at June 30, 2020	62,552,505	1,900	195,316	(63)	1,282,952	(374,098)	(21)	910,670
Balance at December 31, 2020	73,914,844	$ 2,277	195,316	$ (63)	$ 2,235,679	$ (573,576)	$ (83)	$ 1,664,234
Issuance of common shares, net of issuance costs of $5.4 million	1,353,121	45	—	—	222,130	—	—	222,175
Vesting of restricted shares	109,355	3	—	—	—	—	—	3
Exercise of vested options, net of issuance costs of $1.5 million	342,051	15	—	—	9,769	—	—	9,784
Purchase of common stock under ESPP	11,257	—	—	—	751	—	—	751
Stock-based compensation expense	—	—	—	—	22,092	—	—	22,092
Other comprehensive loss	—	—	—	—	—	—	(378)	(378)
Net loss	—	—	—	—	—	(113,163)	—	(113,163)
Balance at March 31, 2021	75,730,628	$ 2,340	195,316	$ (63)	$ 2,490,421	$ (686,739)	$ (461)	$ 1,805,498
Issuance of common shares	—	—	—	—	—	—	—	—
Vesting of restricted shares	3,667	—	—	—	—	—	—	—
Exercise of vested options, net of issuance costs of $0.4 million	344,158	12	—	—	10,897	—	—	10,909
Stock-based compensation expense	—	—	—	—	28,331	—	—	28,331
Other comprehensive loss	—	—	—	—	—	—	(168)	(168)
Net income	—	—	—	—	—	759,225	—	759,225
Balance at June 30, 2021	76,078,453	$ 2,352	195,316	$ (63)	$ 2,529,649	$ 72,486	$ (629)	$ 2,603,795

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)

	Six Months Ended June 30,	
	2021	**2020**
Operating activities:		
Net income (loss)	$ 646,062	$ (149,387)
Reconciliation of net income (loss) to net cash provided by (used in) operating activities:		
Depreciation and amortization	6,368	4,283
Equity-based compensation	50,423	29,848
Other income, non-cash	4,417	889
Changes in:		
Accounts receivable	(6)	66
Prepaid expenses and other assets	(9,470)	23,595
Accounts payable and accrued expenses	13,427	13,216
Deferred revenue	(271)	(201)
Operating lease assets and liabilities	1,259	(111)
Other liabilities, net	(5,870)	(3,652)
Net cash provided by (used in) operating activities	706,339	(81,454)
Investing activities:		
Purchase of property, plant and equipment	(35,473)	(6,596)
Purchases of marketable securities	(715,982)	—
Maturities of marketable securities	289,921	—
Net cash used in investing activities	(461,534)	(6,596)
Financing activities:		
Proceeds from issuance of common shares, net of issuance costs	213,267	83,021
Proceeds from exercise of options and ESPP contributions, net of issuance costs	21,167	7,616
Net cash provided by financing activities	234,434	90,637
Effect of exchange rate changes on cash	10	(28)
Increase (decrease) in cash	479,249	2,559
Cash, cash equivalents and restricted cash, beginning of period	1,185,468	948,812
Cash, cash equivalents and restricted cash, end of period	$ 1,664,717	$ 951,371
Supplemental disclosure of non-cash investing and financing activities		
Property and equipment purchases in accounts payable and accrued expenses	$ 7,533	$ 2,585
Equity issuance costs in accounts payable and accrued expenses	$ 402	$ 1,009

	As of June 30,	
Reconciliation to amounts within the condensed consolidated balance sheets	**2021**	**2020**
Cash and cash equivalents	$ 1,646,646	$ 945,068
Restricted cash	18,071	6,303
Cash, cash equivalents and restricted cash at end of period	1,664,717	951,371

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

1. Basis of Presentation and Significant Accounting Policies

Basis of Presentation

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

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. 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 six-month interim periods ended June 30, 2021 and 2020.

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, 2020, which are contained in the 2020 Annual Report on Form 10-K filed with the Securities and Exchange Commission, or the SEC, on February 16, 2021.

Use of Estimates

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

Significant Accounting Policies

The significant accounting policies used in preparation of these condensed consolidated financial statements for the three and six months ended June 30, 2021 are consistent with those discussed in Note 2 to the consolidated financial statements in the Company's 2020 Annual Report on Form 10-K filed with the SEC on February 16, 2021.

New Accounting Pronouncements – Recently Adopted

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

2. Marketable Securities

The following table summarizes cash equivalents and marketable securities held at June 30, 2021 and December 31, 2020 (in thousands), which are recorded at fair value. The table below excludes $980.6 million and $395.1 million of cash at June 30, 2021 and December 31, 2020, respectively.

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
June 30, 2021				
Cash equivalents:				
Money market funds	$ 583,540	$ —	$ —	$ 583,540
Corporate debt securities	12,487	—	(2)	12,485
Certificates of deposit	21,005	—	—	21,005
Commercial paper	48,996	—	—	48,996
Total cash equivalents	666,028	—	(2)	666,026
Marketable securities:				
U.S. Treasury securities	—	—	—	—
Corporate debt securities	801,145	85	(770)	800,460
Certificates of deposit	44,041	—	—	44,041
Government-sponsored enterprise securities	5,121	1	—	5,122
Commercial paper	93,177	—	—	93,177
Total marketable securities	943,484	86	(770)	942,800
Total cash equivalents and marketable securities	$ 1,609,512	$ 86	$ (772)	$ 1,608,826
December 31, 2020				
Cash equivalents:				
Money market funds	$ 742,958	$ —	$ —	$ 742,958
Corporate debt securities	2,526	1	(24)	2,503
Certificates of deposit	12,527	—	—	12,527
Commercial paper	15,549	—	—	15,549
Total cash equivalents	773,560	1	(24)	773,537
Marketable securities:				
U.S. Treasury securities	47,976	3	—	47,979
Corporate debt securities	324,569	43	(156)	324,456
Certificates of deposit	25,162	—	—	25,162
Government-sponsored enterprise securities	33,738	5	(2)	33,741
Commercial paper	90,375	—	—	90,375
Total marketable securities	521,820	51	(158)	521,713
Total cash equivalents and marketable securities	$ 1,295,380	$ 52	$ (182)	$ 1,295,250

As of June 30, 2021 and December 31, 2020, the aggregate fair value of marketable securities that were in an unrealized loss position for less than twelve months was $622.6 million and $280.3 million, respectively. As of June 30, 2021 and December 31, 2020, no marketable securities were in an unrealized loss position for more than twelve months. The Company has recorded a net unrealized loss of $0.2 million and $0.6 million during the three and six months ended June 30, 2021, respectively, related to its debt securities, which is included in comprehensive loss on the condensed consolidated statements of operations and comprehensive loss. No unrealized losses related to debt securities were recorded in net income (loss) during the three and six months ended June 30, 2021.

The Company determined that there is no material credit risk associated with the above investments as of June 30, 2021. The Company has the intent and ability to hold such securities until recovery. As a result, the Company did not record any charges for credit-related impairments for its marketable securities for the three and six months ended June 30, 2021. No available-for-sale debt securities held as of June 30, 2021 had remaining maturities greater than two years.

3. Fair Value Measurements

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the fair value hierarchy classification of such fair values as of June 30, 2021 and December 31, 2020 (in thousands):

	Fair Value Measurements at June 30, 2021			
	Total	**Level 1**	**Level 2**	**Level 3**
Cash and cash equivalents:				
Cash	$ 980,620	$ 980,620	$ —	$ —
Money market funds	583,540	583,540	—	—
Corporate debt securities	12,485	—	12,485	—
Certificates of deposit	21,005	—	21,005	—
Commercial paper	48,996	—	48,996	—
Marketable securities:				
U.S. Treasury securities	—	—	—	—
Corporate debt securities	800,460	—	800,460	—
Certificates of deposit	44,041	—	44,041	—
Government-sponsored enterprise securities	5,122	—	5,122	—
Commercial paper	93,177	—	93,177	—
Other non-current assets	2,212	—	—	2,212
Total	$ 2,591,658	$ 1,564,160	$ 1,025,286	$ 2,212

	Fair Value Measurements at December 31, 2020			
	Total	**Level 1**	**Level 2**	**Level 3**
Cash and cash equivalents:				
Cash	$ 395,083	$ 395,083	$ —	$ —
Money market funds	742,958	742,958	—	—
Corporate debt securities	2,503	—	2,503	—
Certificates of deposit	12,527	—	12,527	—
Commercial paper	15,549	—	15,549	—
Marketable securities:				
U.S. Treasury securities	47,979	—	47,979	—
Corporate debt securities	324,456	—	324,456	—
Certificates of deposit	25,162	—	25,162	—
Government-sponsored enterprise securities	33,741	—	33,741	—
Commercial paper	90,375	—	90,375	—
Other non-current assets	600	—	—	600
Total	$ 1,690,933	$ 1,138,041	$ 552,292	$ 600

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

The Company holds equity securities classified as Level 3 which are not material to the Company's financial position.

4. Property and Equipment, net

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

	As of	
	June 30, 2021	December 31, 2020
Computer equipment	$ 1,375	$ 727
Furniture, fixtures and other	3,420	3,416
Laboratory equipment	31,090	25,353
Leasehold improvements	25,745	25,473
Construction work in process	41,300	8,366
Total property and equipment, gross	102,930	63,335
Accumulated depreciation	(27,516)	(21,175)
Total property and equipment, net	$ 75,414	$ 42,160

Depreciation expense for the three and six months ended June 30, 2021 was $3.6 million and $6.3 million, respectively. Depreciation expense for the three and six months ended June 30, 2020 was $2.2 million and $4.3 million, respectively.

5. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	As of	
	June 30, 2021	December 31, 2020
Payroll and employee-related costs	$ 15,325	$ 22,402
Research costs	34,023	21,684
Licensing fees	150	1,401
Professional fees	3,085	1,670
Intellectual property costs	6,941	3,625
Accrued property and equipment	7,127	2,835
Other	450	165
Total	$ 67,101	$ 53,782

6. Commitments and Contingencies

Leases

Refer to Note 7 to the consolidated financial statements in the Company's 2020 Annual Report on Form 10-K filed with the SEC on February 16, 2021 for discussion on the Company's lease arrangements. In the second quarter of 2021, the following events results in material changes to the Company's leasing disclosure:

In July 2020, the Company entered into a lease agreement for an office and laboratory facility in Boston, Massachusetts, or the 2020 Lease. The lease expires in October 2034, and the Company has the option to extend the 2020 lease for two additional five-year periods. The 2020 Lease commenced in the second quarter of 2021, and at lease commencement, the Company recorded a right-of-use asset of $149.8 million and a corresponding operating lease liability of $147.9 million. Tenant incentives of $49.2 million were recorded as a reduction to the operating lease asset and liability at lease commencement. The right-of-use asset and corresponding lease liability does not include the additional five-year periods under the renewal option as the Company is not reasonably certain to exercise that option.

In May 2016, the Company entered into a sublease agreement for its primary office and research facility in Cambridge, Massachusetts, or the 2016 Sublease. In December 2019, Casebia Therapeutics, Limited Liability Partnership, or Casebia, became a wholly-owned subsidiary of the Company. In connection therewith, Casebia assigned its sublease for an office and research facility in Cambridge, Massachusetts, or the 2019 Sublease, to the Company. The Company modified its 2016 Sublease and 2019 Sublease such the Company expects to vacate the premises on or about July 2022. The right-of-use assets and right-of-use liabilities have been adjusted accordingly.

Litigation

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

Letters of Credit

As of June 30, 2021, the Company had restricted cash of $18.1 million, representing letters of credit securing the Company's obligations under certain leased facilities. The letters of credit are secured by cash held in a restricted depository account. The cash deposit is recorded in restricted cash in the accompanying condensed consolidated balance sheet as of June 30, 2021.

Research, Manufacturing, License and Intellectual Property Agreements

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

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

Under certain circumstances and if certain contingent future events occur, Vertex Pharmaceuticals Incorporated and certain of its subsidiaries, or Vertex, is eligible to receive up to $395.0 million in potential specified research, development, regulatory and commercial milestones and tiered single-digit percentage royalties on future net sales related to a specified target under an amendment to the 2015 Collaboration Agreement defined in Note 7 below. In addition, Vertex has the option to conduct research at their own cost in certain defined areas that, if beneficial to the CTX001 program and ultimately achieves regulatory approval, then the Company could owe Vertex certain milestone payments aggregating to high eight digits, subject to certain limitations on the profitability of the CTX001 program. Refer to Note 7 for further discussion on the Company's arrangements with Vertex.

7. Significant Contracts

Agreements with Vertex Pharmaceuticals Incorporated and certain of its subsidiaries

Summary

On October 26, 2015, the Company entered into a strategic collaboration, option and license agreement, or the 2015 Collaboration Agreement, with Vertex. The 2015 Collaboration Agreement is focused on the use of the Company's CRISPR/Cas9 gene-editing technology to discover and develop potential new treatments aimed at the underlying genetic causes of human disease.

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

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

In June 2019, the Company and Vertex entered into a series of agreements, which closed on July 23, 2019, including a strategic collaboration and license agreement, or the 2019 Collaboration Agreement, for the development and commercialization of products for the treatment of Duchenne muscular dystrophy, or DMD, and Myotonic Dystrophy Type 1, or DM1. Under the terms of the 2019 Collaboration Agreement, the Company received an upfront, nonrefundable payment of $175.0 million. In addition, the Company is eligible to receive potential aggregate payments of up to $825.0 million based upon the successful achievement of specified research, development, regulatory and commercial milestones for the DMD and DM1 programs. The Company is also eligible to receive tiered royalties on future net sales on any products that may result from this collaboration. For the DMD program, Vertex is responsible for all research, development, manufacturing and commercialization activities and all related costs. For the DM1 program, the Company will perform specified guide RNA research and Vertex is responsible for all other research, development, manufacturing and commercialization costs. Upon Investigational New Drug, or IND, application filing, the Company has the option to forego the DM1 milestones and royalties and, instead, co-develop and co-commercialize all DM1 products globally in exchange for payment of 50% of research and development costs incurred by Vertex from the effective date of the agreement through IND filing.

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

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

On April 16, 2021, the Company and Vertex agreed to amend and restate the JDA and entered into an Amended and Restated Joint Development and Commercialization Agreement, or the “A&R JDCA,” pursuant to which the parties agreed to, among other things, (a) adjust the governance structure for the collaboration and adjust the responsibilities of each party thereunder, whereby Vertex shall lead and have all decision making (i.e., control) in relation to the CTX001 program prospectively; (b) adjust the allocation of net profits and net losses between the parties with respect to CTX001 only, which will be allocated 40% to the Company and 60% to Vertex, prospectively; and (c) exclusively license (subject to the Company’s reserved rights to conduct certain activities) certain intellectual property rights to Vertex relating to the specified product candidates and products (including CTX001) that may be researched, developed, manufactured and commercialized on a worldwide basis under such agreement. The transaction contemplated by the A&R JDCA closed on June 2, 2021. The Company will provide certain specified transition services to Vertex in connection with the agreement.

In connection with the closing of the transaction contemplated by the A&R JDCA, the Company received a $900.0 million up-front payment from Vertex. Additionally, the Company is eligible to receive a one-time $200.0 million milestone payment upon receipt by Vertex of the first marketing approval of the initial product candidate from the U.S. Food and Drug Administration or the European Commission. With respect to CTX001 only, the net profits and net losses, as applicable, incurred under the A&R JDCA through July 1, 2021 in connection with the initial shared product (i.e., CTX001) will be shared equally between the Company and Vertex, and beginning July 1, 2021, the net profits and net losses, as applicable, incurred under the A&R JDCA will be allocated 40% to the Company and 60% to Vertex.

Accounting for the Vertex Agreements

The 2015 Collaboration Agreement, Amendment No. 1, and JDA are collectively the "2015 Agreements" and the 2019 Collaboration Agreement and Amendment No. 2. are collectively the "2019 Agreements." The 2015 Collaboration Agreement, Amendment No. 1, Amendment No. 2, JDA, A&R JDCA and 2019 Collaboration Agreement are collectively the "Vertex Agreements."

The Vertex Agreements include components of a customer-vendor relationship as defined under ASC 606, *Revenue from Contracts with Customers*, or ASC 606, collaborative arrangements as defined under ASC 808, *Collaborative Agreements*, or ASC 808, and research and development costs as defined under ASC 730, *Research and Development*, or ASC 730. Additionally, the A&R JDCA allows the Company to defer a portion of its share of costs under the arrangement if spending on the CTX001 program exceeds specified amounts, up to a maximum per year.

Accounting Analysis Under ASC 606

Accounting for the A&R JDCA

Identification of the Contract

The A&R JDCA represented a contractual modification to the JDA. For accounting purposes, the A&R JDCA was treated as a separate contract.

Identification of Performance Obligations

The Company concluded the A&R JDCA contained a single material promise, an exclusive worldwide license granting Vertex an additional 10% economic interest in the CTX001 program and the right to control development and commercialization of CTX001, or the "CTX001 Exclusive License." The Company concluded the CTX001 Exclusive License was both capable of being distinct and distinct within the context of the A&R JDCA, and the CTX001 Exclusive License was sold at its estimated standalone selling price, or "ESSP." As such, the CTX001 Exclusive License represented a separate performance obligation.

Determination of Transaction Price

The transaction price was comprised of the upfront payment of $900.0 million. The Company determined that all other possible variable consideration resulting from milestones and royalties discussed above was fully constrained at the time of the transaction. The Company will reevaluate the transaction price in each reporting period.

Allocation of Transaction Price to Performance Obligations

The selling price of the performance obligation was determined based on the Company's ESSP. The Company developed the ESSP for the CTX001 Exclusive License 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 ESSP for the exclusive worldwide license for CTX001 was determined to be approximately $900.0 million. The ESSP was determined based on 10% of the probability and present value adjusted cash flows from projected worldwide net profit for CTX001 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. As the Company determined the CTX001 Exclusive License was the only performance obligation, the entire transaction price was allocated to the CTX001 Exclusive License. The aforementioned ESSP reflects the level of risk and expected probability of success inherent in the nature of the associated research area.

Recognition of Revenue

The Company determined that the CTX001 Exclusive License represented functional intellectual property, as the intellectual property provides Vertex with the ability to perform a function or task in the form of research and development, manufacturing and commercialization. As such, the revenue related to the CTX001 Exclusive License was recognized at the point in time, which was upon transfer in the second quarter of 2021.

Accounting for the 2019 Agreements

Identification of the Contract

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

Identification of Performance Obligations

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

Determination of Transaction Price

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

The Company determined that all other possible variable consideration resulting from milestones and royalties discussed above was fully constrained as of June 30, 2021. 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 ESSP. The Company developed the ESSP for all the performance obligations included in the Vertex Agreements with the objective of determining the price at which it would sell such an item if it were to be sold regularly on a standalone basis. The Company then allocated the transaction price to each performance obligation on a relative standalone selling price basis.

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

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

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

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

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

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

Recognition of Revenue

The Company determined that the DMD License and DM1 License represent functional intellectual property, as the intellectual property provides Vertex with the ability to perform a function or task in the form of research and development. As such, the revenue related to the licenses was recognized at the point in time in which they were delivered during the third quarter of 2019.

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

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

The Company recognizes revenue related to the DM1 R&D Services over time as the services are rendered, which was originally expected to be over an 18-month period from the effective date of the 2019 Agreements and is now expected to be over a 24-month period from the effective date of the 2019 Agreements.

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

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

Identification of the Contract

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

Identification of Performance Obligations

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

Determination of Transaction Price

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

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

Allocation of Transaction Price to Performance Obligations

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

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

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

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

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

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

Recognition of Revenue

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

Revenue recognized in connection with the Vertex Agreements

Revenue recognized under the Vertex Agreements for the three and six months ended June 30, 2021, respectively, was $900.2 million and $900.4 million, respectively. Revenue recognized under the Vertex Agreements for the three and six months ended June 30 2020, respectively, was not material.

As of June 30, 2021, there was no current deferred revenue related to the collaboration with Vertex. As of December 31, 2020, there was $ 0.4 million of current deferred revenue related to the collaboration with Vertex. As of June 30, 2021, there was $11.8 million of non-current deferred revenue related to the collaboration with Vertex, which is unchanged from December 31, 2020. The transaction price allocated to the remaining performance obligations was $11.8 million.

Future Milestones under the Vertex Agreements

The Company has evaluated the milestones that may be received in connection with the Vertex Agreements. As discussed above, the Company is eligible to receive up to $410.0 million in additional development, regulatory and commercial milestones and royalties on net product sales for each of the three collaboration targets that Vertex licensed in the fourth quarter of 2019. Each milestone is payable only once per collaboration target, regardless of the number of products directed to such collaboration target that achieve the relevant milestone event.

The Company is eligible to receive additional potential future payments of up to $800.0 million based upon the successful achievement of specified research, development, regulatory and commercial milestones for the DMD and DM1 programs. The Company is also eligible to receive tiered royalties on future net sales on any products that may result from this collaboration; however, the Company has the option to forego the DM1 milestones and royalties to co-develop and co-commercialize all DM1 products globally.

The Company is eligible to receive additional potential future payments of up to $200.0 million upon receipt by Vertex of the first marketing approval of the initial product candidate from the U.S. Food and Drug Administration or the European Commission. In addition, the Company has the option to conduct research at their own cost in certain defined areas that, if beneficial to the CTX001 program and CTX001 ultimately achieves regulatory approval in such areas, then the Company could be entitled to certain milestone payments aggregating to high eight digits from Vertex.

Each of the remaining milestones are fully constrained as of June 30, 2021. There is uncertainty that the events to obtain the research and developmental milestones will be achieved given the nature of clinical development and the stage of the CRISPR/Cas9 technology. The remaining research, development and regulatory milestones will be constrained until it is probable that a significant revenue reversal will not occur. Commercial milestones and royalties relate predominantly to a license of intellectual property and are determined by sales or usage-based thresholds. The commercial milestones and royalties are accounted for under the royalty recognition constraint and will be accounted for as constrained variable consideration. The Company applies the royalty recognition constraint for each commercial milestone and will not recognize revenue for each until the subsequent sale of a licensed product (achievement of each) occurs.

Accounting Analysis under ASC 808

In connection with the Vertex Agreements, the Company identified the following collaborative elements, which are accounted for under ASC 808: (i) development and commercialization services for shared products, including any transition services related to CTX001, as stipulated in the A&R JDCA, the cost of such services will be shared by the parties pursuant to the cost share agreement; (ii) R&D Services for follow-on products; and (iii) committee participation. The related impact of the cost sharing associated with research and development is included in research and development expense. Expenses related to services performed by the Company are classified as research and development expense. Payments received from Vertex for partial reimbursement of expenses are recorded as a reduction of research and development expense.

During the three and six months ended June 30, 2021, the Company recognized $26.9 million and $46.9 million of research and development expense related to the Vertex Agreements, respectively. During the three and six months ended June 30, 2020, the Company recognized $9.9 million and $19.0 million of research and development expense related to the Vertex Agreements, respectively. Research and development expense for the three and six months ended June 30, 2021 was net of $12.6 million and $23.2 million of reimbursements from Vertex, respectively. Research and development expense for the three and six months ended June 30, 2020 was net of $5.5 million and $11.0 million of reimbursements from Vertex, respectively.

Accounting Analysis under ASC 730

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

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

In connection with the *A&R JDCA,* Vertex has the option to conduct research at their own cost in certain defined areas that, if beneficial to the CTX001 program and CTX001 ultimately achieves regulatory approval in such areas, then the Company could owe Vertex certain milestone payments aggregating to high eight digits, subject to certain limitations on the profitability of the CTX001 program.

Agreements with Bayer Healthcare LLC

Summary

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

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

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

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

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

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

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

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

terminate the 2019 Option Agreement upon the Company's bankruptcy or insolvency, or for convenience at any time, after giving written notice.

Accounting Analysis

Accounting for the 2015 Casebia Agreements

Transactions under the 2015 Casebia Agreements ceased on the effective date of the 2019 Casebia Agreements. There was no financial impact of the 2015 Casebia Agreements for the three and six months ended June 30, 2021 and 2020.

Accounting for the 2019 Casebia Agreements

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

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

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

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

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

During the three and six months ended June 30, 2021, the Company recorded a benefit of $2.7 million and $7.0 million, respectively, to research and development expense for qualifying expenses incurred under the 2019 Option Agreement. During the three and six months ended June 30, 2020, the Company recorded a benefit of $2.3 million and $4.2 million, respectively, to research and development expense for qualifying expenses incurred under the 2019 Option Agreement. As of June 30, 2021 and December 31, 2020, the Company has recorded $0.0 million and $7.0 million, respectively, in other current liabilities relating to certain research and development obligations to be satisfied within one year of the balance sheet date. As of June 30, 2021, the Company has recorded $4.8 million in other long-term liabilities consisting of the previously allocated value of such obligations to be satisfied beyond one year from the balance sheet date as well as the relative fair value of the options, which is unchanged from December 31, 2020.

8. Share Capital

The Company had 145,364,335 authorized common shares as of June 30, 2021, with a par value of CHF 0.03 per share. Share Capital consisted of the following:

		As of	
Type of Share Capital	**Conditional Capital**	**June 30, 2021**	**December 31, 2020**
Common shares	Registered share capital	80,321,227	75,133,951
Common shares	Authorized share capital	39,316,975	17,625,426
Common shares	Conditional share capital - Bonds or similar debt instruments	4,919,700	4,919,700
Common shares	Conditional share capital - Employee benefit plans	20,806,433	17,493,709
	Total	145,364,335	115,172,786

At-the-Market Offerings

In August 2019, the Company entered into an Open Market Sale Agreement℠ with Jefferies under which the Company was able to offer and sell, from time to time at its sole discretion through Jefferies, as its sales agent, its common shares, par value of CHF 0.03 per share, or the August 2019 Sales Agreement. In August 2019, the Company filed a prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to $200.0 million, or the 2019 ATM. During the year ended December 31, 2020, the Company issued and sold an aggregate of 2.2 million common shares under the 2019 ATM at an average price of $89.47 per share for aggregate proceeds of $195.5 million, which were net of equity issuance costs of $4.5 million.

In December 2020, in connection with the August 2019 Sales Agreement, the Company filed a prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to $350.0 million, or the 2020 ATM. During the year ended December 31, 2020, the Company issued and sold an aggregate of 1.8 million common shares under the 2020 ATM at an average price of $169.57 per share for aggregate proceeds of $298.0 million, which were net of equity issuance costs of $4.5 million. Additional equity issuance costs for stamp taxes related to shares sold in 2020 related to the 2019 ATM and 2020 ATM were $4.9 million, of which $4.0 million was payable as of December 31, 2020.

In January 2021, the Company issued and sold under the 2020 ATM an aggregate of 0.3 million common shares at an average price of $162.46 per share with aggregate proceeds of $46.7 million, which were net of equity issuance costs of $0.7 million. An additional $0.5 million of stamp taxes on this amount was paid in 2021.

In January 2021, in connection with the August 2019 Sales Agreement, the Company filed a prospectus supplement with the SEC to offer and sell, from time to time, common shares having aggregate gross proceeds of up to $600.0 million, or the 2021 ATM. As of June 30, 2021, the Company has issued and sold an aggregate of 1.1 million common shares under the 2021 ATM at an average price of $169.82 per share for aggregate proceeds of $177.8 million, which were net of equity issuance costs of $2.4 million. An additional $1.8 million of stamp taxes on this amount was paid in 2021.

July 2020 Offering

In July 2020, the Company sold 7.4 million common shares through an underwritten public offering (inclusive of shares sold pursuant to the exercise of the underwriters' option to purchase additional shares) at a public offering price of $70.00 per share for aggregate net proceeds of $484.8 million, which were net of equity issuance costs and stamp tax of $32.5 million.

9. Stock-based Compensation

During the three and six months ended June 30, 2021 and 2020, the Company recognized the following stock-based compensation expense (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Research and development	$ 16,195	$ 8,021	$ 29,040	$ 15,383
General and administrative	12,136	7,676	21,383	14,465
Total	$ 28,331	$ 15,697	$ 50,423	$ 29,848

Stock option activity

The following table summarizes stock option activity for the six months ended June 30, 2021:

	Shares	Weighted-average exercise price per share
Outstanding at December 31, 2020	8,101,980	$ 42.44
Granted	1,074,458	135.81
Exercised	(686,548)	32.97
Cancelled or forfeited	(141,351)	54.14
Outstanding at June 30, 2021	8,348,539	$ 55.03
Exercisable at June 30, 2021	4,141,831	$ 34.08
Vested and expected to vest at June 30, 2021	8,348,539	$ 55.03

As of June 30, 2021, total unrecognized compensation expense related to stock options was $183.6 million, which the Company expects to recognize over a remaining weighted-average period of 2.9 years.

Restricted stock activity

The following table summarizes restricted stock activity for the six months ended June 30, 2021:

	Restricted Stock	Weighted-Average Grant Date Fair Value
Unvested balance as of December 31, 2020	894,092	$ 70.55
Granted	306,395	134.58
Vested	(113,022)	40.28
Cancelled or forfeited	(39,099)	71.99
Unvested balance as of June 30, 2021	1,048,366	$ 92.47

As of June 30, 2021, total unrecognized compensation expense related to unvested restricted common shares was $69.3 million, which the Company expects to recognize over a remaining weighted-average vesting period of 2.7 years.

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

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Net income (loss)	$ 759,225	$ (79,656)	$ 646,062	$ (149,387)
Basic weighted-average common shares outstanding	75,826,594	61,420,746	75,418,160	61,134,214
Effect of potentially dilutive securities:				
Outstanding options	4,149,901	—	4,519,827	—
Unvested restricted common shares	473,462	—	520,868	—
Diluted weighted-average common shares outstanding	80,449,956	61,420,746	80,458,855	61,134,214
Basic net income (loss) per common share	10.01	(1.30)	8.57	(2.44)
Diluted net income (loss) per common share	9.44	(1.30)	8.03	(2.44)

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Outstanding options	1,936,503	8,197,261	1,483,205	8,197,261
Unvested restricted common shares	385,550	923,366	203,335	923,366
ESPP	10,594	13,509	7,946	13,509
Total	2,332,647	9,134,136	1,694,486	9,134,136

11. Income Taxes

During the three and six months ended June 30, 2021, the Company recorded an income tax provision of $4.1 million and $4.7 million, respectively, representing an effective tax rate of 0.5% and 0.7%, respectively. During the three and six months ended June 30, 2020, the Company recorded an income tax provision of $0.4 million and $0.8 million, respectively, representing an effective tax rate of -0.5% and -0.5%, 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, research credits generated, and the valuation allowance recorded against certain deferred tax assets. The Company maintains a valuation allowance against certain deferred tax assets that are not more-likely-than-not realizable.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with (i) our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and (ii) our audited consolidated financial statements and related notes and management's discussion and analysis of financial condition and results of operations included in our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the Securities and Exchange Commission, or the SEC, on February 16, 2021. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and impact and potential impacts on our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including, without limitation, those factors set forth in the "Risk Factors" section of our Annual Report on Form 10-K for the year ended December 31, 2020 and the "Risk Factors" section of subsequent Quarterly Reports on Form 10-Q, our actual results or timing of certain events could differ materially from the results or timing described in, or implied by, these forward-looking statements.

Special Note About Coronavirus (COVID-19)

Since March 2020, we have been evaluating the actual and potential business impacts related to the outbreak of a novel strain of virus named SARS-CoV-2 (severe acute respiratory syndrome 2), or coronavirus, which causes coronavirus disease, or COVID-19. As a result of the coronavirus pandemic, we have experienced, and may further experience, disruptions, pauses and/or delays that have and could further adversely impact our business operations, and/or associated timelines. As we gradually return to work in accordance with state and local regulations, we maintain temporary work-from-home procedures for all employees other than for those personnel and contractors who perform essential activities that must be completed on-site. If negative developments relating to the coronavirus pandemic continue, including as a result of a continued so-called "resurgence" or additional "waves", we may be required to restrict on-site staff at our offices and laboratories again and at times have limited access to our offices on a temporary and intermittent basis; with respect to our hemoglobinopathies clinical trials, we may elect to pause patient dosing in certain of our trials again if ICU beds and related healthcare resources become significantly constrained again or governmental authorities impose additional business or travel restrictions; with respect to our immuno-oncology clinical trials, investigators participating in our clinical trials may not want to take the risk of exposing cancer patients to the coronavirus since the dosing of patients is conducted within an in-patient setting; and certain aspects of our supply chain could be disrupted if our third party suppliers and manufacturers paused their operations again in response to such negative developments and/or as a result of national and local regulations. The ultimate impact of the coronavirus pandemic on our business operations remains uncertain and subject to change and will depend on future developments, which cannot be accurately predicted. We will continue to monitor the situation closely.

Overview

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

Our Programs

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

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

We and Vertex are investigating CTX001 in an ongoing Phase 1/2 open-label clinical trial, CLIMB THAL-111, that is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 12 to 35 with TDT. In the fourth quarter of 2019, we expanded the TDT patient population for CTX001 to include beta zero/beta zero subtypes. The first two patients in the trial were treated sequentially and, following data from the initial two patients indicating successful engraftment and an acceptable safety

profile, the trial opened for concurrent dosing. CLIMB THAL-111 is designed to follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up study. CTX001 has been granted Regenerative Medicine Advanced Therapy, or RMAT, designation, as well as Fast Track Designation and Rare Pediatric Disease designation by the U.S. Food and Drug Administration, or FDA, for the treatment of TDT. In addition, CTX001 for the treatment of TDT has received orphan drug designation, or ODD, by the FDA and European Commission; and CTX001 has been granted Priority Medicines (PRIME) designation by the European Medicines Agency for the treatment of TDT. In the second quarter of 2021, at the European Hematology Association Annual Meeting, we released updated clinical data from the first fifteen patients with TDT treated with CTX001 who had reached at least three months of follow-up after CTX001 dosing.

We and Vertex are also investigating CTX001 in an ongoing Phase 1/2 open-label clinical trial, CLIMB SCD-121, that is designed to assess the safety and efficacy of a single dose of CTX001 in patients ages 12 to 35 with severe SCD. Similar to the trial in TDT, the first two patients in the trial were treated sequentially and, following data from the initial two patients indicating successful engraftment and an acceptable safety profile, the trial opened for concurrent dosing. CLIMB SCD-121 is designed to follow patients for approximately two years after infusion. Each patient will be asked to participate in a long-term follow-up study. CTX001 has been granted RMAT Designation, as well as Fast Track Designation and Rare Pediatric Disease designation by the FDA for the treatment of SCD. In addition, CTX001 for the treatment of SCD has received ODD by the FDA and European Commission; and CTX001 has been granted Priority Medicines (PRIME) designation by the European Medicines Agency for the treatment of SCD. In the second quarter of 2021, at the European Hematology Association Annual Meeting, we released updated clinical data from the first seven patients with SCD treated with CTX001 who had reached at least three months of follow-up after CTX001 dosing.

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

CTX110. Our lead immuno-oncology product candidate, CTX110, is a healthy donor-derived gene-edited allogeneic CAR-T investigational therapy targeting cluster of differentiation 19, or CD19. CTX110 is being investigated in an ongoing Phase 1 single-arm, multi-center, open-label clinical trial, CARBON, that is designed to assess the safety and efficacy of several dose levels of CTX110 for the treatment of relapsed or refractory B-cell malignancies. In October 2020, we released initial top-line data from the ongoing CARBON clinical trial.

CTX120. CTX120 is a healthy donor-derived gene-edited allogeneic CAR-T investigational therapy targeting B-cell maturation antigen. CTX120 is being investigated in an ongoing Phase 1 single-arm, multi-center, open-label clinical trial that is designed to assess the safety and efficacy of several dose levels of CTX120 for the treatment of relapsed or refractory multiple myeloma. CTX120 has received ODD by the FDA.

CTX130. CTX130 is a healthy donor-derived gene-edited allogeneic CAR-T investigational therapy targeting cluster of differentiation 70, or CD70, an antigen expressed on various solid tumors and hematologic malignancies. CTX130 is being developed for the treatment of both solid tumors, such as renal cell carcinoma, and T-cell and B-cell hematologic malignancies. CTX130 is being investigated in two ongoing independent Phase 1 single-arm, multi-center, open-label clinical trials that are designed to assess the safety and efficacy of several dose levels of CTX130 for the treatment of relapsed or refractory renal cell carcinoma and various types of lymphoma, respectively. CTX130 for the treatment of T-cell lymphoma has received ODD by the FDA.

Strategic Partnerships

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

Vertex. We established our initial collaboration agreement in 2015 with Vertex, which focused on TDT, SCD, cystic fibrosis and select additional indications. In December 2017, we entered into a joint development and commercialization agreement with Vertex pursuant to which, among other things, we are co-developing and preparing to co-commercialize CTX001 for TDT and SCD. On April 16, 2021 we and Vertex agreed to amend and restate our existing joint development and commercialization agreement, pursuant to which, among other things, we will continue to develop and prepare to commercialize CTX001 for TDT and SCD in partnership with Vertex, subject to, among other things, certain adjustments to the governance structure for the collaboration and responsibilities of the parties. In addition, we adjusted the allocation of net profits and net losses between the parties with respect to CTX001 only and exclusively licensed (subject to our reserved rights to conduct certain activities) certain intellectual property rights to Vertex relating to the specified product candidates and products (including CTX001) that may be researched, developed, manufactured and commercialized under such agreement. We also entered into a strategic collaboration and license agreement with Vertex in June 2019 for the development and commercialization of products for the treatment of Duchenne muscular dystrophy and myotonic dystrophy type 1.

ViaCyte. We entered into the ViaCyte Collaboration Agreement in September 2018 with ViaCyte, Inc., or ViaCyte, to pursue the discovery, development and commercialization of gene-edited allogeneic stem cell therapies for the treatment of diabetes and in July 2021, we entered into a joint development and commercialization agreement with ViaCyte. Under the joint development and commercialization agreement, we and ViaCyte will jointly develop and commercialize product candidates and shared products for use in the treatment of diabetes type 1, diabetes type 2 and insulin dependent/requiring diabetes throughout the world. We believe the combination of ViaCyte's stem cell capabilities and our gene editing capabilities has the potential to enable a beta-cell replacement product that may deliver durable benefit to patients without the need for immune suppression.

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

Financial Overview

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

Our revenue to date has been primarily derived from collaborations with partners. We were profitable for the year ended December 31, 2019 due to collaboration revenue from Vertex and the gain from consolidating Casebia, as well as for the three and six months ended June 30, 2021 due to collaboration revenue from Vertex, but we do not expect to sustain our profitability in future years. With the exception of the year ended December 31, 2019 and the three and six months ended June 30, 2021, we have incurred significant net operating losses each year since our inception and we expect to continue to incur net operating losses for the foreseeable future. As of June 30, 2021, we had $2,589.4 million in cash, cash equivalents and marketable securities and retained earnings of $72.5 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; incur facilities costs associated with such personnel growth; develop manufacturing infrastructure; and incur additional costs associated with operating as a public company. In addition, we expect to spend significantly more on capital expenditures than we have historically incurred in order to construct and build out our new U.S. headquarters for research and development in Boston, Massachusetts, and our cell therapy manufacturing facility in Framingham, Massachusetts. Please refer to Part I, Item 2 of our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the SEC on February 16, 2021 for more information about these facilities.

Revenue

We have not generated any revenue to date from product sales and do not expect to do so in the near future. Revenue recognized for the three and six months ended June 30, 2021 was $900.7 million and $901.2 million, respectively. Revenue recognized for the three and six months ended June 30, 2020 was not material. For additional information about our revenue recognition policy, see Note 2, "Summary of Significant Accounting Policies," in our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the SEC on February 16, 2021, as well as Note 7 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 collaboration with Vertex and ViaCyte, as well as in connection with the Bayer Transaction, we do not track research and development costs on a program-by-program basis.

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

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, and potential commercialization of our product candidates. In addition, we anticipate increased expenses related to the reimbursements of third-party patent related expenses in connection with certain of our in-licensed intellectual property.

Other Income (Expense), Net

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

Results of Operations

Comparison of three months ended June 30, 2021 and 2020 (in thousands):

	Three Months Ended June 30,		Period to Period Change
	2021	2020	
Revenue:			
Collaboration revenue	$ 900,202	$ 44	$ 900,158
Grant revenue	499	—	499
Total revenue	900,701	44	900,657
Operating expenses:			
Research and development	108,277	59,380	48,897
General and administrative	29,806	21,353	8,453
Total operating expenses	138,083	80,733	57,350
Income (loss) from operations	762,618	(80,689)	843,307
Other income, net	750	1,412	(662)
Net income (loss) before income taxes	763,368	(79,277)	842,645
Provision for income taxes	(4,143)	(379)	(3,764)
Net income (loss)	$ 759,225	$ (79,656)	$ 838,881

Collaboration Revenue

Collaboration revenue for the three months ended June 30, 2021 was $900.2 million. Collaboration revenue for the three months ended June 30, 2020, was not material. The increase in collaboration revenue was due to a $900.0 million upfront payment in connection with the A&R JDCA with Vertex. Please refer to Note 7 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 $108.3 million for the three months ended June 30, 2021, compared to $59.4 million for the three months ended June 30, 2020. The increase of approximately $48.9 million was primarily attributable to the following:

- $24.1 million of increased variable research and development costs;
- $15.3 million of increased employee compensation, benefit and other headcount related expenses, of which $8.2 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth; and,
- $8.7 million of increased facility-related expenses.

General and Administrative Expenses

General and administrative expenses were $29.8 million for the three months ended June 30, 2021, compared to $21.4 million for the three months ended June 30, 2020. The increase of approximately $8.5 million was primarily attributable to the following:

- $6.0 million of increased employee compensation, benefit and other headcount related expenses, of which $4.5 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth; and,
- $2.5 million of increased intellectual property costs.

Other Income, Net

Other income was $0.8 million for the three months ended June 30, 2021, compared to $1.4 million of income for the three months ended June 30, 2020. The change was primarily due to a decrease in interest income earned on cash, cash equivalents and marketable securities for the three months ended June 30, 2021 as a result of decreases in interest rates.

Comparison of six months ended June 30, 2021 and 2020 (in thousands):

	Six Months Ended June 30,		Period to Period Change
	2021	2020	
Revenue:			
Collaboration revenue	$ 900,404	$ 201	$ 900,203
Grant revenue	836	—	836
Total revenue	901,240	201	901,039
Operating expenses:			
Research and development	198,842	113,573	85,269
General and administrative	54,323	40,903	13,420
Total operating expenses	253,165	154,476	98,689
Income (loss) from operations	648,075	(154,275)	802,350
Other income, net	2,705	5,644	(2,939)
Net income (loss) before income taxes	650,780	(148,631)	799,411
Provision for income taxes	(4,718)	(756)	(3,962)
Net income (loss)	$ 646,062	$ (149,387)	$ 795,449

Collaboration Revenue

Collaboration revenue for the six months ended June 30, 2021 was $900.4 million. Collaboration revenue for the six months ended June 30, 2020 was not material. The increase in collaboration revenue was due to a $900.0 million upfront payment in connection with the A&R JDCA with Vertex. Please refer to Note 7 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 $198.8 million for the six months ended June 30, 2021, compared to $113.6 million for the six months ended June 30, 2020. The increase of approximately $85.3 million was primarily attributable to the following:

- $41.9 million of increased variable research and development costs;
- $29.2 million of increased employee compensation, benefit and other headcount related expenses, of which $13.7 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth; and,
- $15.0 million of increased facility-related expenses.

General and Administrative Expenses

General and administrative expenses were $54.3 million for the six months ended June 30, 2021, compared to $40.9 million for the six months ended June 30, 2020. The increase of approximately $13.4 million was primarily attributable to the following:

- $9.9 million of increased employee compensation, benefit and other headcount related expenses, of which $6.9 million is increased stock-based compensation expense, primarily due to an increase in headcount to support overall growth; and,
- $3.4 million of increased intellectual property costs.

Other Income, Net

Other income was $2.7 million for the six months ended June 30, 2021, compared to $5.6 million of income for the six months ended June 30, 2020. The change was primarily due to interest income earned on cash, cash equivalents and marketable securities for the six months ended June 30, 2021 as a result of decrease in interest rates

Liquidity and Capital Resources

As of June 30, 2021, we had cash, cash equivalents and marketable securities of approximately $2,589.4 million, of which approximately $1,200.9 million was held outside of the United States.

In August 2019, we entered into a Open Market Sale Agreement℠ with Jefferies LLC, or Jefferies, under which we are able to offer and sell, from time to time at our sole discretion through Jefferies, as our sales agent, our common shares, par value of CHF 0.03 per share, or the August 2019 Sales Agreement. In December 2020, in connection with the August 2019 Sales Agreement, we filed a prospectus supplement with the SEC to offer and sell from time to time common shares having aggregate gross proceeds of up to $350.0 million, or the 2020 ATM. In January 2021, we issued and sold under the 2020 ATM an aggregate of 0.3 million common shares at an average price of $162.46 per share with aggregate proceeds of $46.7 million, which were net of equity issuance costs of $0.7 million. An additional $0.5 million of stamp taxes on this amount was paid in 2021.

In January 2021, in connection with the August 2019 Sales Agreement, we filed a prospectus supplement with the SEC to offer and sell from time to time common shares having aggregate gross proceeds of up to $600.0 million. As of June 30, 2021, we have issued and sold an aggregate of 1.1 million common shares under the 2021 ATM at an average price of $169.82 per share for aggregate proceeds of $177.8 million, which were net of equity issuance costs of $2.4 million. An additional $1.8 million of stamp taxes on this amount was paid in 2021.

We have predominantly incurred losses and cumulative negative cash flows from operations since our inception. We were profitable for the year ended December 31, 2019 due to collaboration revenue from Vertex and the gain from consolidating Casebia, as well as for the three and six months ended June 30, 2021 due to collaboration revenue from Vertex, but we do not expect to sustain our profitability in future years. With the exception of the year ended December 31, 2019 and the three and six months ended June 30, 2021, we have incurred significant net operating losses each year since our inception and we expect to continue to incur net operating losses for the foreseeable future. As of June 30, 2021, we had $2,589.4 million in cash, cash equivalents and marketable securities and retained earnings of $72.5 million. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through public or private equity or debt financings, strategic collaborations, or other sources.

Funding Requirements

Our primary uses of capital are, and we expect will continue to be, research and development activities, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses, patent prosecution filing and maintenance costs for our licensed intellectual property and general overhead costs, including costs associated with operating as a public company. 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 and clinical activities and initiate preclinical studies to support initial drug applications. We also anticipate that we will incur significant capital expenditures as we develop our manufacturing infrastructure and facilities.

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 financings, debt financings and payments received in connection with our collaboration agreements. We are entitled to research payments under our collaboration with Vertex. Additionally, we are eligible to earn payments, in each case, on a per-product basis under our collaboration with Vertex. Except for this source of funding, we do not have any committed external source of liquidity. We intend to consider opportunities to raise additional funds through the sale of equity or debt securities when market conditions are favorable to us to do so. However, including as a result of the coronavirus pandemic, the trading prices for our common shares and other biopharmaceutical companies have been highly volatile. As a result, we may face difficulties raising capital through sales of our common shares or such sales may be on unfavorable terms. In addition, a recession, depression or other sustained adverse market event, including resulting from the spread of the coronavirus, could materially and adversely affect our business and the value of our common shares. To the extent that we raise additional capital through the future sale of equity or debt securities, the ownership interests of our shareholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing shareholders. If we raise additional funds through collaboration arrangements in the future, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Outlook

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

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

Cash Flows

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

	Six Months Ended June 30,		Period to Period Change
	2021	2020	
Net cash provided by (used in) operating activities	$ 706,339	$ (81,454)	$ 787,793
Net cash used in investing activities	(461,534)	(6,596)	(454,938)
Net cash provided by financing activities	234,434	90,637	143,797
Effect of exchange rate changes on cash	10	(28)	38
Net increase decrease in cash	$ 479,249	$ 2,559	$ 476,690

Net Cash Used in Operating Activities

Net cash provided by operating activities was $706.3 million for the six months ended June 30, 2021, compared to cash used in operating activities of $81.5 million for the six months ended June 30, 2020. The $787.8 million increase in cash used in operating activities was primarily driven by an increase in net income of $795.4 million, from a net loss of $149.4 million for the six months ended June 30, 2020 to net income of $646.1 million for the six months ended June 30, 2021. The increase in net income is primarily driven by a $900.0 million upfront payment from Vertex in connection with the A&R JDCA. Additionally, non-cash expense increased $26.2 million, primarily related to an increase in stock compensation and depreciation. The increase was offset by increased spending in our clinical and pre-clinical stage programs and increased payroll and payroll-related expenses to support overall growth, as well as a $33.8 million decrease in net changes of operating assets and liabilities.

Net Cash Used in Investing Activities

Net cash used in investing activities for the six months ended June 30, 2021 was $461.5 million, compared to $6.6 million for the six months ended June 30, 2020. The increase in net cash used in investing activities consisted primarily of purchases of marketable securities and property and equipment.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the six months ended June 30, 2021 was $234.4 million, compared with $90.6 million for the six months ended June 30, 2020. The net cash provided by financing activities for the six months ended June 30, 2021 consisted of option exercise proceeds, net of issuance costs. Additionally, 1.4 million common shares were issued in connection with our Open Market Sale Agreement℠, which resulted in $219.9 million of net cash proceeds, after deducting $3.1 million in equity issuance costs and $2.2 million of stamp taxes.

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, 2020 filed with the SEC on February 16, 2021. There have been no material changes from the contractual commitments and obligations previously disclosed in our Annual Report on Form 10-K.

Off-Balance Sheet Arrangements

As of June 30, 2021, we did not have any off-balance sheet arrangements as defined under applicable SEC rules.

Critical Accounting Policies and Significant Judgments and Estimates

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

We believe that our most critical accounting policies are those relating to revenue recognition, variable interest entities and equity-based compensation, and there have been no changes to our accounting policies discussed in our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the SEC on February 16, 2021.

Recent Accounting Pronouncements

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

Item 3. Qualitative and Quantitative Disclosures about Market Risk

Interest Rate Sensitivity

We are exposed to market risk related to changes in interest rates. As of June 30, 2021, we had cash, cash equivalents and marketable securities of $2,589.4 million, primarily invested in U.S. treasury securities and government agency securities, corporate bonds, commercial paper and money market accounts invested in U.S. government agency securities. Due to the conservative nature of these instruments, we do not believe that we have a material exposure to interest rate risk. If interest rates were to increase or decrease by 1%, the fair value of our investment portfolio would increase or decrease by an immaterial amount.

Foreign Currency Exchange Rate Risk

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

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 June 30, 2021, 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 June 30, 2021, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

During the quarter ended June 30, 2021, there were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

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

There have been no material developments with respect to the legal proceedings previously disclosed in "Item 3. Legal Proceedings" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2020 filed with the SEC on February 16, 2021.

Item 6. Exhibits

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

Exhibit Number	Description of Document
10.1*#	Second Amendment to Lease dated April __, 2021, by and between CRISPR Therapeutics, Inc. and 33 NYA OWNER (DE) LLC, as successor in interest to CRP/KING 33 NY AVE. OWNER, L.L.C.
10.2*†#	Letter Agreement dated April 29, 2021 by and between CRISPR Therapeutics, Inc. and Pfizer Inc.
31.1*	Certification of Chief Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Chief Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1*+	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the inline XBRL document.
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104*	Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101.*)

* Filed herewith.

\+ The certification attached as Exhibit 32.1 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of CRISPR Therapeutics AG under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

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

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

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: July 29, 2021

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

Dated: July 29, 2021

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

Exhibit 10.1

33 New York Avenue
Framingham, MA

Second Amendment to Lease
CRISPR Therapeutics, Inc.

THIS SECOND AMENDMENT TO LEASE ("Second Amendment") is made as of April___, 2021 (the "Effective Date") by and between 33 NYA OWNER (DE) LLC, a Delaware limited liability company ("Landlord"), and CRISPR THERAPEUTICS, INC., a Delaware corporation ("Tenant").

Background

Pursuant to the provisions of that certain Lease dated as of May 5, 2020, as amended by that certain First Amendment dated as of November , 2020 (collectively, the "Original Lease"), Landlord, as successor-in-interest to CRP/KING 33 NY AVE. OWNER, L.L.C., leases to Tenant and Tenant leases from Landlord, those certain premises in the building known and numbered as 33 New York Avenue, Framingham, MA, the "Premises") as set forth in the Original Lease. Capitalized terms used and not defined herein shall have the respective meanings ascribed to them in the Original Lease. The Original Lease, as amended hereby, is hereinafter referred to as the "Lease."

Tenant has requested, a Landlord has approved, a revision to the Plans which will increase the square footage of the Prime Premises by a de minimis amount.

Landlord and Tenant desire to enter into this Second Amendment to provide for such revision of the Prime Premises min accordance with the terms and conditions set forth herein.

Agreement

NOW, THEREFORE, in consideration of the foregoing and mutual covenants contained herein, Landlord and Tenant hereby agree to modify and amend the Original Lease as follows:

1. Prime Premises. Exhibit 1A to the Lease is hereby deleted and replace with Exhibit 1A attached hereto.

(a) Notwithstanding the revision to Exhibit 1A, the parties hereby acknowledge and agree that there shall be no change to the Rentable Square Footage of the Premises, which shall remain 50,249 rentable square feet for all purposes under the Lease.

2. Miscellaneous

(a) Brokerage. Tenant shall indemnify and hold Landlord and its trustees, managers, members, principals, beneficiaries, partners, officers, directors, employees, mortgagees and agents harmless from all claims of any other brokers, agents or finders claiming to have represented Tenant in connection with this Second Amendment. Any assistance rendered by any agent or employee of

Landlord in connection with this Second Amendment or any subsequent amendment or modification or any other document related hereto has been or will be made as an accommodation to Tenant solely in furtherance of consummating the transaction on behalf of Landlord, and not as agent for Tenant.

(b) Tenant Confirmations. Tenant represents and warrants to Landlord that (1) the person(s) executing this Second Amendment on behalf of Tenant are duly authorized and have full power to execute and deliver this Second Amendment, (2) to Tenant's knowledge as of the date hereof, (i) Landlord is not in default of its obligations under the Lease, (ii) nor do circumstances exist which, with the giving of notice or passage of time, or both, would constitute a default by Landlord under the Lease, and (iii) Tenant has no claim, offset, or defense against the enforcement of the Lease in accordance with its terms, and (3) Tenant is not acting, directly or indirectly, for or on behalf of any person, group, entity, or nation named by any Executive Order or the United States Treasury Department as a terrorist, "Specially Designated National and Blocked Person", or other banned or blocked person, group, entity, nation, or transaction pursuant to any law, order, rule, or regulation that is enforced or administered by the Office of Foreign Assets Control and that it is not engaged in this transaction, directly or indirectly, on behalf of, or instigating or facilitating this transaction, directly or indirectly, on behalf of any such person, group, entity, or nation.

(c) General. The submission of this Second Amendment to Tenant or a summary of some or all of its provisions for examination does not constitute a reservation of or option for the Leased Premises or an offer to lease any space, and no legal obligations shall arise with respect to the Leased Premises hereunder or other matters herein unless and until such time as this Second Amendment is executed and delivered by Landlord and Tenant. This Second Amendment may be executed in one or more counterparts and, when executed by each party, shall constitute an agreement binding on all parties notwithstanding that all parties are not signatories to the original or the same counterpart provided that all parties are furnished a copy or copies thereof reflecting the signature of all parties. Transmission of a facsimile or by email of a pdf copy of the signed counterpart of this Second Amendment or execution thereof by electronic signature shall be deemed the equivalent of the delivery of the original.

(d) Entire Amendment. This Second Amendment contains all of the agreements of the parties with respect to the subject matter hereof and supersedes all prior dealings between the parties with respect to such subject matter.

(e) Binding Amendment. This Second Amendment shall be binding upon, and shall inure to the benefit of, the parties hereto and their respective successors and assigns.

(f) Governing Law. This Second Amendment shall be governed by the laws of the Commonwealth of Massachusetts without regard to conflict of laws principles.

(g) Authority. Landlord and Tenant each warrants to the other that the person or persons executing this Second Amendment on its behalf has or have authority to do so and that such execution has fully obligated and bound such party to all of the terms and provisions of this Second Amendment.

(h) Ratification. Except as expressly modified by this Second Amendment, the Lease is hereby confirmed and shall remain in full force and effect.

[*signature page follows*]

IN WITNESS WHEREOF, Landlord and Tenant have entered into this Second Amendment as a sealed Massachusetts instrument as of the Effective Date set forth above.

LANDLORD:

33 NYA OWNER (DE) LLC,
a Delaware limited liability company

By: /s/ Brian Barriero
Name: Brian Barriero
Title: Vice President

By: /s/ Kristen Binck
Name: Kristen Binck
Title: Vice President

TENANT:

CRISPR THERAPEUTICS, INC.,
a Delaware corporation

By: /s/ Denis Desmond
Name: Denis Desmond
Title: Head of Facilities Services, Operations & Real Estate

EXHIBIT 1A
LEASE PLAN – PRIME PREMISES

[***]

Exhibit 10.2

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

April 29, 2021

VIA E-MAIL

Pfizer Inc.
Legal Division
235 East 42nd Street New York, NY 10017
Attention: William C. Longa

Re: Sublease dated August 1, 2016 ("5th Floor Sublease") between Pfizer Inc. ("Pfizer") and CRISPR Therapeutics, Inc. ("CRISPR") of the 5th floor of Premises at 610 Main Street, Cambridge, MA (the "Building") and Sublease dated May 6, 2016 ("6th-7th Floor Sublease") between Pfizer and CRISPR of the 6th and 7th floors of the Building (collectively, the "Subleases")

Dear William:

This letter is sent to confirm and memorialize our discussions regarding CRISPR's proposed further sublease of the above-referenced premises, as more fully detailed in our letter to you dated [***] (the "Sublease Consent Letter"). As provided in the Sublease Consent Letter, CRISPR proposes to further sublease the [***] floor premises to [***] (the "[***] Sub-Sublease") and further sublease [***] floor premises to [***] (the "[***] Sub-Sublease", and collectively with the [***] Sublease, the "Proposed Sub-Subleases").

Notwithstanding anything to the contrary contained in the Subleases, Pfizer and CRISPR have agreed as follows with respect to the Proposed Sub-Subleases only:

1. CRISPR hereby waives the thirty (30) day requirement set forth in Section 19(b) of the Subleases in which Pfizer may respond to the Sublease Consent Letter. Notwithstanding anything in the Subleases to the contrary, Pfizer may consent, refuse consent, or elect to exercise its recapture rights with respect to the premises covered by each Proposed Sub-Sublease and terminate the applicable Sublease, in each case, each as and to the extent permitted under the applicable Sublease and this letter agreement, by providing written notice to CRISPR no later than [***] (the parties acknowledging that Pfizer may elect to

treat each of the Proposed Sub-Subleases separately), and Pfizer's failure to provide any such written notice with respect to a Proposed Sub-Sublease by such date shall be deemed to constitute Pfizer's consent to such Proposed Sub-Sublease;

2. CRISPR may, in its discretion, continue to negotiate the Proposed Sub-Subleases and may execute the Proposed Sub-Subleases, subject to the rights of Pfizer under the Subleases and this letter agreement and the rights of Overlandlord (as defined in the Subleases). Pfizer shall not be responsible for any costs or expenses incurred in connection with such negotiations and/or signing, even if consent to such Proposed Sub-Subleases is subsequently refused pursuant to the applicable Sublease;

3. If Pfizer shall elect to exercise its recapture rights with respect to one or both of the Subleases and to terminate the applicable Sublease(s), the effective date of any such termination shall be July 1, 2022 (the "Termination Date"), subject to clause (4) below, as though such date were the original Expiration Date of the applicable Sublease(s) for all purposes;

4. Notwithstanding the Termination Date as provided in clause (3) and notwithstanding anything to the contrary set forth in the Subleases, Pfizer acknowledges and agrees that CRISPR may adjust the Termination Date upon not less than six (6) months prior written notice to Pfizer to a date (i) not more than three (3) months prior to July 1, 2022 or (b) not more than three (3) months after July 1, 2022; and

5. The foregoing terms and conditions shall modify the Subleases solely with respect to the Proposed Sub-Subleases and except as otherwise modified by this letter agreement, the Subleases shall remain in full force and effect and unamended.

Kindly acknowledge your agreement to the foregoing terms by signing this letter in the appropriate acknowledgement section below and returning a copy to me by email. Please call if you have any questions.

Sincerely,
CRISPR THERAPEUTICS, INC.

/s/ Michael Tomsicek
Name: Michal Tomsicek
Title: CFO

/s/ Denis Desmond
Denis Desmond
Head of Facilities Services, Operations & Real Estate

Acknowledged and agreed:
PFIZER INC.

/s/ Gary Annino
Name: Gary Annino
Title: Sr Director, FS & CRE

Exhibit 31.1

CERTIFICATIONS

I, Samarth Kulkarni, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: July 29, 2021

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

Exhibit 31.2

CERTIFICATIONS

I, Michael Tomsicek, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: July 29, 2021

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

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of CRISPR Therapeutics AG (the "Company") for the period ended June 30, 2021 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)

July 29, 2021

/s/ Michael Tomsicek

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

July 29, 2021