## **UNITED STATES** SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

## **FORM 10-Q**

(Mark One)

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

For the quarterly period ended March 31, 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 001-39208** 

## **Beam Therapeutics Inc.**

(Exact name of Registrant as specified in its Charter)

Delaware (State or other jurisdiction of incorporation or organization)

81-5238376 (I.R.S. Employer Identification No.)

26 Landsdowne Street

Cambridge, MA (Address of principal executive offices) 02139

(Zip Code)

Registrant's telephone number, including area code: (857) 327-8775

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

	Trading	Name of each exchange
Title of each class	Symbol(s)	on which registered
Common Stock, par value \$0.01 per share	BEAM	Nasdaq Global Select Market

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

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

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

Large accelerated filer

Non-accelerated filer

	Accelerated filer
X	Smaller reporting company
	Emerging growth company

X

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.

Γ

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

The number of shares of registrant's common stock outstanding as of May 4, 2021 was 62,578,078

#### NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such forward-looking statements reflect, among other things:

- our current expectations and anticipated results of operations;
- our expectations regarding the initiation, timing, progress and results of our research and development programs and preclinical and clinical studies, including our intention to file an Investigational New Drug, or IND, application for BEAM-101 during the second half of 2021, our plans to initiate IND-enabling studies for BEAM-102 and BEAM-201 during 2021, and our belief that we are on track to nominate our first development candidate from our liver portfolio in the second half of 2021;
- our ability to develop a sustainable portfolio;
- our ability to create a hub for partnering with other companies;
- our plans for pre-clinical studies for product candidates in our pipeline;
- our ability to advance any product candidates that we may develop and successfully complete any clinical studies, including the manufacture of any such product candidates;
- our ability to pursue a broad suite of clinically validated delivery modalities;
- our expectations regarding our ability to generate additional novel LNPs that we believe could accelerate novel nonviral delivery of gene editing
  payloads to tissues beyond the liver and our ability to expand the reach of gene editing, including as a result of our acquisition of Guide
  Therapeutics;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- developments related to our competitors and our industry;
- the expected timing, progress and success of our collaborations with third parties and our ability to identify and enter into future license agreements and collaborations;
- developments related to base editing technologies;
- our ability to successfully develop our three distinct pipelines and obtain and maintain approval for our product candidates;
- our ability to successfully establish and maintain a commercial-scale current Good Manufacturing Practice, or cGMP, manufacturing facility and that this facility will be operational in 2023;
- regulatory developments in the United States and foreign countries;
- our ability to attract and retain key scientific and management personnel;
- our expectations regarding the strategic and other potential benefits of our acquisition of Guide Therapeutics, and
- the impact of the coronavirus disease of 2019, or COVID-19, pandemic on our business.

All of these statements are subject to known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements, market trends, or industry results to differ materially from those expressed or implied by such forward-looking statements. Therefore, any statements contained herein that are not statements of historical fact may be forward-looking statements and should be evaluated as such. Without limiting the foregoing, the words "anticipate," "expect," "suggest," "plan," "believe," "intend," "project," "forecast," "estimates," "targets," "projections," "should," "could," "may," "might," "will," and the negative thereof and similar words and expressions are intended to identify forward-looking statements. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in "Risk Factors" in Part II, Item 1A of this report. Unless legally required, we assume no obligation to update any such forward-looking information to reflect actual results or changes in the factors affecting such forward-looking information.

When we use the terms "Beam," the "Company," "we," "us" or "our" in this Quarterly Report on Form 10-Q, we mean Beam Therapeutics Inc. and its subsidiaries on a consolidated basis, unless the context indicates otherwise.

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#### PART I. FINANCIAL INFORMATION

#### Beam Therapeutics Inc. Condensed Consolidated Balance Sheets (*Unaudited*) (in thousands, except share and per share amounts)

		March 31, 2021	December 31, 2020
Assets			
Current assets:			
Cash and cash equivalents	\$	97,241	\$ 162,171
Marketable securities		406,238	137,500
Prepaid expenses and other current assets		11,101	8,650
Total current assets		514,580	 308,321
Property and equipment, net		51,994	38,513
Restricted cash		14,840	14,840
Operating lease right-of-use assets		106,794	86,859
Other assets		5,033	3,144
Total assets	\$	693,241	\$ 451,677
Liabilities and stockholders' equity	-		
Current liabilities:			
Accounts payable	\$	7,983	\$ 6,314
Accrued expenses and other current liabilities		14,976	18,487
Derivative liabilities		73,100	71,200
Current portion of lease liability		3,885	4,218
Current portion of equipment financing liability		2,142	2,118
Total current liabilities		102,086	 102,337
Long-term lease liability		125,825	96,014
Long-term equipment financing liability		4,741	5,294
Contingent consideration liabilities		36,818	—
Other liabilities		1,342	2,471
Total liabilities		270,812	 206,116
Commitments and contingencies (See Note 7, <i>Leases</i> , Note 9, <i>License agreements</i> and Note 10, <i>Collaboration and license agreements</i> )			
Stockholders' equity:			
Preferred stock, \$0.01 par value; 25,000,000 shares authorized, and no shares issued or outstanding at March 31, 2021 and December 31, 2020, respectively		_	_
Common stock, \$0.01 par value; 250,000,000 shares authorized, 62,528,153 and 58,446,016 issued,			
and 61,735,119 and 57,254,178 outstanding at March 31, 2021 and December 31, 2020, respectively		617	573
Additional paid-in capital		1,021,032	642,633
Accumulated other comprehensive loss		(24)	(9)
Accumulated deficit		(599,196)	 (397,636)
Total stockholders' equity		422,429	 245,561
Total liabilities, redeemable convertible preferred stock, and stockholders' equity	\$	693,241	\$ 451,677

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

#### Beam Therapeutics Inc. Condensed Consolidated Statements of Operations and Other Comprehensive Loss (*Unaudited*) (in thousands, except share and per share amounts)

		arch 31,		
		2021		2020
License revenue	\$	6	\$	6
Operating expenses:				
Research and development		190,106		21,549
General and administrative		10,273		6,812
Total operating expenses		200,379		28,361
Loss from operations		(200,373)		(28,355)
Other income (expense):				
Change in fair value of derivative liabilities		(1,900)		(2,700)
Change in fair value of contingent consideration liabilities		(305)		—
Interest and other income, net		1,018		597
Total other income (expense)		(1,187)		(2,103)
Net loss	\$	(201,560)	\$	(30,458)
Unrealized loss on marketable securities		(15)		(360)
Comprehensive loss	\$	(201,575)	\$	(30,818)
Reconciliation of net loss to net loss attributable to common stockholders:				
Net loss	\$	(201,560)	\$	(30,458)
Accretion of redeemable convertible preferred stock to redemption value, including dividends on				
preferred stock				(1,277)
Net loss attributable to common stockholders	\$	(201,560)	\$	(31,735)
Net loss per common share attributable to common stockholders, basic and diluted	\$	(3.35)	\$	(1.03)
Weighted-average common shares used in net loss per share attributable to common stockholders,				
basic and diluted		60,210,120		30,725,077
		,		

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

#### Beam Therapeutics Inc. Condensed Consolidated Statements of Redeemable Convertible Preferred Stock and Stockholders' (Deficit) Equity (Unaudited) (in thousands, except share amounts)

	Redeemable C Preferred	Stock	Commo	n Stock	Additional Paid-in	Accumulated Other Comprehensive	Accumulated	Total Stockholders' (Deficit)
	Shares	Amount	Shares	Amount	Capital	Loss	Deficit	Equity
Balance at December 31, 2019	130,616,784	\$ 302,049	7,326,185	\$ 73	\$ 1,851	\$ 16	\$ (203,044)	\$ (201,104)
Accretion of redeemable convertible preferred stock to redemption value	_	1,277	_	—	(1,277)	—	_	(1,277)
Conversion of redeemable convertible preferred stock to common stock upon closing of initial public offering	(130,616,784)	(303,326)	29,127,523	291	303,035	_	_	303,326
Issuance of common stock from initial public offering, net of issuance costs of \$18.7 million	_	_	12,176,471	122	188,201	—	_	188,323
Vesting of restricted common stock	_	_	387,866	4	(4)		_	_
Stock-based compensation	_	_	_	_	2,792	_	_	2,792
Exercise of common stock options	_		59,305	1	151	_	_	152
Other comprehensive loss			_	_		(360)	_	(360)
Net loss	_	_		_	—		(30,458)	(30,458)
Balance at March 31, 2020		\$	49,077,350	\$ 491	\$ 494,749	\$ (344)	\$ (233,502)	\$ 261,394

#### Beam Therapeutics Inc. Condensed Consolidated Statements of Redeemable Convertible Preferred Stock and Stockholders' (Deficit) Equity - Continued (Unaudited) (in thousands, except share amounts)

	Commo	n Stoc	k	1	Additional Paid-in		ccumulated Other mprehensive	Ac	ccumulated	Sto	Total ckholders'
	Shares	A	Amount		Capital	Loss			Deficit		Equity
Balance at December 31, 2020	57,254,178	\$	573	\$	642,633	\$	(9)	\$	(397,636)	\$	245,561
Issuance of common stock from private placement, net of issuance costs of \$8.0 million	2,795,700		28		251,977		_		_		252,005
Issuance of common stock to acquire Guide	1,087,153		10		120,022		_		_		120,032
Vesting of restricted common stock	398,804		4		(4)		_		_		_
Stock-based compensation			_		4,648		_		_		4,648
Exercise of common stock options	199,284		2		1,756						1,758
Other comprehensive loss			_		_		(15)		_		(15)
Net loss	_		_		—				(201,560)		(201, 560)
Balance at March 31, 2021	61,735,119	\$	617	\$	1,021,032	\$	(24)	\$	(599,196)	\$	422,429

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

#### Beam Therapeutics Inc. Condensed Consolidated Statements of Cash Flows (Unaudited) (in thousands)

		Three Months Er	,		
		2021	2020		
Operating activities	<b>A</b>		¢ (D0.45)		
Net loss	\$	(201,560)	\$ (30,458		
Adjustments to reconcile net loss to net cash used in operating activities:		1 2 2 2			
Depreciation and amortization		1,398	1,10		
Amortization of investment discount (premiums)		15	(129		
In-process research and development charge		154,953			
Stock-based compensation expense		4,648	2,792		
Change in operating lease right-of-use assets		2,359	952		
Non-cash research and development license expense, net		—	264		
Change in fair value of derivative liabilities		1,900	2,700		
Change in fair value of contingent consideration liabilities		305	-		
Other		(976)	_		
Changes in operating assets and liabilities:					
Prepaid expenses and other current assets		(2,365)	(3,559		
Other long-term assets		(185)	(7)		
Accounts payable		(666)	(47)		
Accrued expenses and other liabilities		(4,458)	378		
Operating lease liabilities		6,111	(879		
Other long-term liabilities		(57)	()		
Net cash used in operating activities		(38,578)	(27,393		
Investing activities					
Purchases of property and equipment		(11,478)	(3,040		
Purchases of marketable securities		(289,218)	(117,719		
Maturities of marketable securities		20,450	44,723		
Net cash acquired from Guide		620	_		
Purchase of long-term investment		_	(750		
Net cash used in investing activities		(279,626)	(76,792		
Financing activities					
Proceeds from initial public offering, net of underwriting discount		_	192,510		
Proceeds from offering of common stock		260,000	_		
Payment of equity offering costs		(7,955)	(958		
Proceeds from equipment financings		_	1,62		
Repayment of equipment financings		(529)	(315		
Proceeds from exercise of stock options		1,758	152		
Net cash provided by financing activities		253,274	193,014		
Net change in cash, cash equivalents and restricted cash		(64,930)	88,829		
Cash, cash equivalents and restricted cash—beginning of period		177,011	50,553		
Cash, cash equivalents and restricted cash—end of period	\$	112,081	\$ 139,382		

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

#### Beam Therapeutics Inc. Condensed Consolidated Statements of Cash Flows - Continued (Unaudited) (in thousands)

Three Months Ended March 31,			
	2021		2020
\$	159	\$	134
\$		\$	303,326
\$	6,671	\$	1,032
\$	23,366	\$	4,032
\$	250	\$	707
\$	36,513	\$	_
\$	120,032	\$	
\$	_	\$	1,277
	\$ \$ \$ \$ \$ \$ \$ \$	2021 \$ 159 \$	2021       \$     159       \$     \$       \$     6,671       \$     23,366       \$     250       \$     36,513

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

#### Beam Therapeutics Inc. Notes to Condensed Consolidated Financial Statements (Unaudited)

#### 1. Nature of the business and basis of presentation

#### Organization

Beam Therapeutics Inc. (the "Company" or "Beam") is a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. Beam's vision is to provide life-long cures to patients suffering from genetic diseases. The Company was incorporated on January 25, 2017 (Inception) as a Delaware corporation and began operations in July 2017. Its principal offices are in Cambridge, Massachusetts.

In February 2021, the Company entered into an Agreement and Plan of Merger ("the Guide Merger Agreement") to acquire Guide Therapeutics, or Guide. Pursuant to the Guide Merger Agreement, the Company paid Guide's former stockholders and optionholders upfront consideration in an aggregate amount of \$120.0 million, excluding customary purchase price adjustments, in shares of its common stock, based upon the volume-weighted average price of the common stock over the ten trading day period ending on February 19, 2021. In addition, Guide's former stockholders and optionholders will be eligible to receive up to an additional \$100.0 million in technology and \$220.0 million in product milestone payments, payable in the Company's common stock.

#### Liquidity and capital resources

Since its inception, the Company has devoted substantially all of its resources to building its base editing platform and advancing development of its portfolio of programs, establishing and protecting its intellectual property, conducting research and development activities, organizing and staffing the Company, business planning, raising capital and providing general and administrative support for these operations. The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry including, but not limited to, technical risks associated with the successful research, development and manufacturing of product candidates, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Current and future programs will require significant research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

In February 2020, the Company completed its initial public offering, or IPO, in which the Company issued and sold 12,176,471 shares of its common stock, including 1,588,235 shares pursuant to the full exercise of the underwriters' option to purchase additional shares, at a public offering price of \$17.00 per share, for aggregate gross proceeds of \$207.0 million. The Company received approximately \$188.3 million in net proceeds after deducting underwriting discounts and offering expenses payable by the Company. In connection with the IPO, all outstanding shares of the Company's redeemable convertible preferred stock converted into 29,127,523 shares of its common stock.

In October 2020, the Company issued and sold 5,750,000 shares of its common stock, including 750,000 shares pursuant to the full exercise of the underwriters' option to purchase additional shares, at a public offering price of \$23.50 per share, for aggregate gross proceeds of \$135.1 million. The Company received approximately \$126.6 million in net proceeds after deducting underwriting discounts and offering expenses payable by the Company.

On January 16, 2021, the Company entered into a Securities Purchase Agreement with certain purchasers, pursuant to which the Company agreed to sell and issue to the purchasers, in a private placement, shares of common stock of the Company. The closing of the private placement occurred on January 21, 2021. The Company issued and sold 2,795,700 shares of its common stock at a purchase price of \$93.00 per share for aggregate gross proceeds of \$260.0 million, before deducting fees to the placement agents and other offering expenses payable by the Company (See Note 11, *Preferred stock and common stock*). The Company received approximately \$252.0 million in net proceeds after deducting fees to the placement agents and offering expenses payable by the Company.

Since its inception, the Company has incurred substantial losses and had an accumulated deficit of \$599.2 million as of March 31, 2021. The Company expects to generate operating losses and negative operating cash flows for the foreseeable future.

The Company expects that its cash, cash equivalents, and marketable securities as of March 31, 2021 of \$503.5 million will be sufficient to fund its operations for at least the next twelve months from the date of issuance of these financial statements. The Company will need additional financing to support its continuing operations and pursue its growth strategy. Until such time as the Company can generate significant revenue from product sales, if ever, it expects to finance its operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. The Company may be unable to raise additional funds or enter into such other agreements when needed on favorable terms or at all. The inability to raise capital as and when needed would have a negative impact on the Company's financial condition and its ability to pursue its business strategy. The Company will need to generate significant revenue to achieve profitability, and it may never do so.

#### COVID-19-related significant risks and uncertainties

With the ongoing concern related to the COVID-19 pandemic during 2020 and in the first three months of 2021, the Company has maintained and expanded its business continuity plans to address and mitigate the impact of the COVID-19 pandemic on its business. In March 2020, to protect the health of its employees, and their families and communities, the Company restricted access to its offices to personnel who performed critical activities that must be completed on-site, limited the number of such personnel that could be present at its facilities at any one time, and requested that most of its employees work remotely. In May 2020, as certain states eased restrictions, the Company established new protocols to better allow its full laboratory staff access to the Company's facilities. These protocols included several shifts working over a seven-day-week protocol. The Company expects to continue incurring additional costs to ensure it adheres to the guidelines instituted by the Centers for Disease Control and Prevention, or CDC, and to provide a safe working environment to its onsite employees.

The extent to which the COVID-19 pandemic impacts the Company's business, its corporate development objectives, results of operations and financial condition, and the value of and market for its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements, and the effectiveness of actions taken globally to contain and treat the disease. Disruptions to the global economy, disruption of global healthcare systems, and other significant impacts of the COVID-19 pandemic could have a material adverse effect on the Company's business, financial condition, results of operations and growth prospects.

While the COVID-19 pandemic did not significantly impact the Company's business or results of operations during the three months ended March 31, 2021, the length and extent of the pandemic, its consequences, and containment efforts will determine the future impact on the Company's operations and financial condition.

#### 2. Summary of significant accounting policies

The Company's significant accounting policies are disclosed in the audited consolidated financial statements for the year ended December 31, 2020, and notes thereto, which are included in the Company's Annual Report on Form 10-K that was filed with the Securities and Exchange Commission on March 15, 2021. Since the date of those financial statements, there have been no material changes to Beam's significant accounting policies except as noted below.

#### **Basis of presentation**

The accompanying condensed consolidated financial statements have been prepared in accordance with United States generally accepted accounting principles, or GAAP. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification, or ASC, and Accounting Standards Update, or ASU, of the Financial Accounting Standards Board, or FASB.

#### Principles of consolidation

The accompanying condensed consolidated financial statements include the results of operations of the Company and its wholly-owned subsidiaries. All intercompany transactions and balances have been eliminated in consolidation.

#### Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities and expenses, the determination of the fair value equity instruments and intangible assets acquired in an asset acquisition, and the disclosure of contingent assets and liabilities as of and during the reporting period. The Company bases its estimates and assumptions on historical experience, when available, and on various factors that it believes to be reasonable under the circumstances. The Company evaluates its estimates and assumptions on an ongoing basis. Actual results could differ from these estimates.

#### Cash, cash equivalents, and restricted cash

Cash and cash equivalents consist of standard checking accounts, money market accounts, and all highly liquid investments with a remaining maturity of three months or less at the date of purchase. Restricted cash represents collateral provided for letters of credit issued as security deposits in connection with the Company's leases of its corporate and manufacturing facilities.



The following table reconciles cash, cash equivalents, and restricted cash reported within the Company's condensed consolidated balance sheets to the total of the amounts shown in the condensed consolidated statements of cash flows (in thousands):

	I	March 31, 2021	1	March 31, 2020
Cash and cash equivalents	\$	97,241	\$	126,050
Restricted cash		14,840		13,332
Total cash, cash equivalents, and restricted cash	\$	112,081	\$	139,382

#### **Asset Acquisitions**

In 2018, the Company adopted ASU 2017-01, *Business Combinations*, or ASU 2017-01, which clarified the definition of a business. The Company measures and recognizes asset acquisitions that are not deemed to be business combinations based on the cost to acquire the assets, which includes transaction costs, and the consideration is allocated to the items acquired based on a relative fair value methodology. Goodwill is not recognized in asset acquisitions. In an asset acquisition, the cost allocated to acquire in-process research and development with no alternative future use is charged to research and development expense at the acquisition date.

At the time of acquisition, the Company determines if a transaction should be accounted for as a business combination or acquisition of assets.

#### **Contingent Consideration Liabilities**

The estimated fair value of contingent consideration liabilities, initially measured and recorded on the acquisition date, are considered to be a Level 3 instrument and are reviewed quarterly, or whenever events or circumstances occur that indicate a change in fair value. The contingent consideration liabilities are recorded at fair value at the end of each reporting period with changes in estimated fair values recorded in other income (expense) in the condensed consolidated statements of operations.

The estimated fair value is determined based on probability adjusted discounted cash flow models that include significant estimates and assumptions pertaining to technology and product development. Significant changes in any of the probabilities of success would result in a significantly higher or lower fair value measurement. Significant changes in the probabilities as to the periods in which milestones will be achieved would result in a significantly lower or higher fair value measurement.

#### Recent accounting pronouncements

In November 2018, the FASB issued ASU 2018-18, *Collaborative Arrangements*, or ASU 2018-18, which clarifies certain transactions between collaborative arrangement participants should be accounted for as revenue when the collaborative arrangement participant is a customer in the context of a unit of account and precludes recognizing as revenue consideration received from a collaborative arrangement participant if the participant is not a customer. ASU 2018-18 became effective for the Company on January 1, 2021. As such the Company adopted ASU 2018-18 effective January 1, 2021 using a retrospective adoption date of January 1, 2018 when the Company adopted ASU 2014-09, *Revenue from Contracts with Customers (Topic 606)* and its related amendments, or, collectively, ASC 606. The adoption of ASU 2018-18 did not have a material impact on the Company's consolidated financial statements.

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses* (Topic 326): *Measurement of Credit Losses on Financial Instruments*, or ASU 2016-13, which requires the measurement and recognition of expected credit losses for financial assets held at amortized cost. ASU 2016-13 replaces the existing incurred loss impairment model with an expected loss model. It also eliminates the concept of other-than-temporary impairment and requires credit losses related to available-for-sale debt securities to be recorded through an allowance for credit losses rather than as a reduction in the amortized cost basis of the securities. These changes may result in earlier recognition of credit losses. The Company adopted ASU 2016-13 on January 1, 2021. The adoption of ASU 2016-13 did not have a material impact on the Company's consolidated financial statements.

#### 3. Property and equipment, net

Property and equipment consist of the following (in thousands):

	March 31, 2021	De	ecember 31, 2020
Lab equipment	\$ 20,506	\$	17,201
Leasehold improvements	12,706		12,706
Furniture and fixtures	1,080		1,078
Computer equipment	576		547
Construction in process	 27,379		15,880
Total property and equipment	 62,247		47,412
Less accumulated depreciation	(10,253)		(8,899)
Property and equipment, net	\$ 51,994	\$	38,513

Depreciation expense for the three months ended March 31, 2021 and 2020 was \$1.4 million and \$1.1 million, respectively.

#### 4. Fair value of financial instruments

The Company's financial instruments that are measured at fair value on a recurring basis consist of cash equivalents, marketable securities, contingent consideration liabilities related to the Guide Merger and success payment derivative liabilities pursuant to the license agreement between Harvard University, or Harvard, and the Company, or the Harvard License Agreement, and the license agreement between Broad Institute of MIT and Harvard, or Broad Institute, and Blink, or the Broad License Agreement.

The Company also holds investments in privately issued corporate equity securities, which are accounted for as investments in equity securities. These investments do not have readily determinable fair values and the Company values such investments based on the cost of the equity securities adjusted for observable market transactions or impairments, if any. As of March 31, 2021, the Company held \$3.6 million of investments in privately issued corporate equity securities. During the three months ended March 31, 2021, as a result of an observable market transaction (Level 2), the Company adjusted the value of one of its investments and recorded an unrealized gain of \$1.0 million in interest and other income (expense), net in the Company's condensed consolidated statements of operations and other comprehensive loss.

The following table sets forth the fair value of the Company's financial assets and liabilities by level within the fair value hierarchy at March 31, 2021 (in thousands):

	Carrying amount	Fair value	Level 1 Level 2		Level 2		Level 2		Level 3
<u>Assets</u>									
Cash equivalents:									
Money market funds	\$ 62,831	\$ 62,831	\$ 62,831	\$	—	\$	—		
Commercial paper	33,054	33,053			33,053		_		
Marketable securities:									
Commercial paper	377,611	377,611			377,611		_		
Corporate notes	23,623	23,623			23,623		_		
Government securities	5,004	5,004	—		5,004		—		
Total assets	\$ 502,123	\$ 502,122	\$ 62,831	\$	439,291	\$	_		
<u>Liabilities</u>									
Success payment liability – Harvard	\$ 36,500	\$ 36,500	\$ _	\$	_	\$	36,500		
Success payment liability – Broad Institute	36,600	36,600	_				36,600		
Contingent consideration liability - Technology	29,648	29,648	\$ _	\$	_	\$	29,648		
Contingent consideration liability - Product	7,170	7,170	_		_		7,170		
Total liabilities	\$ 109,918	\$ 109,918	\$ 	\$		\$	109,918		



The following table sets forth the fair value of the Company's financial assets and liabilities by level within the fair value hierarchy at December 31, 2020 (in thousands):

	Carrying amount	Fair value	Level 1	Level 2	Level 3
<u>Assets</u>					
Cash equivalents:					
Money market funds	\$ 88,259	\$ 88,259	\$ 88,259	\$ —	\$ —
Commercial paper	60,494	60,497		60,497	_
Corporate notes	12,314	12,308	—	12,308	
Marketable securities:					
Commercial paper	113,622	113,622	—	113,622	
Corporate notes	7,836	7,836		7,836	_
U.S. Treasury securities	11,009	11,009		11,009	_
Government securities	5,033	5,033		5,033	_
Total assets	\$ 298,567	\$ 298,564	\$ 88,259	\$ 210,305	\$ _
<u>Liabilities</u>					
Success payment liability – Harvard	\$ 35,500	\$ 35,500	\$ _	\$ _	\$ 35,500
Success payment liability – Broad Institute	35,700	35,700	_	_	35,700
Total liabilities	\$ 71,200	\$ 71,200	\$ 	\$ 	\$ 71,200

**Cash equivalents** – Money market funds included within cash equivalents are classified within Level 1 of the fair value hierarchy because they are valued using quoted market prices in active markets. Commercial paper and corporate notes are classified within Level 2 of the fair value hierarchy because pricing inputs are other than quoted prices in active markets, which are either directly or indirectly observable as of the reporting date, and fair value is determined through the use of models or other valuation methodologies.

**Marketable securities** – The Company measures its marketable securities at fair value on a recurring basis and classify those instruments within Level 2 of the fair value hierarchy. Marketable securities are classified within Level 2 of the fair value hierarchy because pricing inputs are other than quoted prices in active markets, which are either directly or indirectly observable as of the reporting date, and fair value is determined using models or other valuation methodologies.

Success Payment Liabilities – As discussed further in Note 9, *License agreements*, the Company is required to make payments determined based upon the achievement of specified multiples of the initial weighted average value of the Company's Series A Preferred Stock, or, subsequent to the IPO, the market value of Beam's common stock, at specified valuation dates. The Company's liability for success payments under the Harvard License Agreement and Broad License Agreement are carried at fair value. To determine the estimated fair value of the success payment liability, the Company uses a Monte Carlo simulation methodology, which models the future movement of stock prices based on several key variables.

The following variables were incorporated in the calculation of the estimated fair value of the Harvard and Broad Institute success payment liabilities:

		Harvard			Broad Institu			itute		
	Ν	March 31, 2021					March 31, 2021		D	ecember 31, 2020
Fair value of common stock (per share)	\$	80.04	\$	81.64	\$	80.04	\$	81.64		
Expected volatility		76%		74%		75%		74%		
Expected term (years)		0.10-8.25		0.35-8.49		0.10-9.11		0.35-9.36		

The computation of expected volatility was estimated using available information about the historical volatility of stocks of similar publicly traded companies for a period matching the expected term assumption. In addition, the Company incorporated the estimated number, timing, and probability of valuation measurement dates in the calculation of the success payment liability.

The following table reconciles the change in the fair value of success payment liabilities based on Level 3 inputs (in thousands):

	Three Months Ended March 31, 2021						
	Harvard Broad Institute Total					Total	
Balance at December 31, 2020	\$	35,500	\$	35,700	\$	71,200	
Change in fair value		1,000		900		1,900	
Balance at March 31, 2021	\$	36,500	\$	36,600	\$	73,100	

**Contingent Consideration Liabilities** – As discussed further in Note 8, *Guide Acquisition*, under the Guide Merger Agreement, Guide's former stockholders and optionholders are eligible to receive up to an additional \$100.0 million in technology and \$220.0 million in product milestone payments, payable in the Company's common stock valued using the volume-weighted average price of the Company's stock over the ten-day trading period ending two trading days prior to the date on which the applicable milestone is achieved. As these milestones are payable in the Company's common stock, the milestone payments result in liability classification under ASC 480, *Distinguishing Liabilities from Equity*. These contingent consideration liabilities are carried at fair value which was estimated by applying a probability-based model, which utilized inputs that were unobservable in the market. These contingent consideration liabilities are classified within Level 3 of the fair value hierarchy. The fair value of the contingent consideration liabilities as of March 31, 2021 is \$36.8 million.

The following table reconciles the change in fair value of the contingent consideration liabilities based on level 3 inputs (in thousands):

	 Three Months Ended March 31, 2021							
	echnology Ailestones	Produc	t Milestones		Total			
Balance at February 23, 2021 (inception)	\$ 29,403	\$	7,110	\$	36,513			
Change in fair value	245		60		305			
Balance at March 31, 2021	\$ 29,648	\$	7,170	\$	36,818			

The following variables were incorporated in the calculation of the estimated fair value of the Guide technology and product contingent consideration liabilities:

	Technology Milestones	Product Milestones
	March 31, 2021	March 31, 2021
Discount Rate	8.50%	8.50%
Probability of Payment	10-60%	2-15%
Projected Year of Payment	2021-2022	2023-2027

#### 5. Marketable securities

The following table summarizes the Company's marketable securities held at March 31, 2021 (in thousands):

	Am	ortized Cost	U	Gross nrealized Gains	τ	Gross Jnrealized Losses	F	Fair Value
Commercial paper	\$	377,625	\$	14	\$	(28)	\$	377,611
Corporate notes		23,633				(10)		23,623
Government securities		5,004		_		_		5,004
Total	\$	406,262	\$	14	\$	(38)	\$	406,238

The following table summarizes the Company's marketable securities held at December 31, 2020 (in thousands):

	Amo	ortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	\$	113,628	\$ 11	\$ (17)	\$ 113,622
Corporate notes		7,839	2	(5)	7,836
U.S Treasury securities		11,009	_	_	11,009
Government securities		5,033	—	—	5,033
Total	\$	137,509	\$ 13	\$ (22)	\$ 137,500

The amortized cost of marketable securities is adjusted for amortization of premiums and accretion of discounts to maturity. At March 31, 2021, the balance in accumulated other comprehensive loss was comprised solely of activity related to marketable securities. There were no realized gains or losses recognized on the sale or maturity of marketable securities for the three months ended March 31, 2021 and 2020 and, as a result, the Company did not reclassify any amounts out of accumulated other comprehensive loss for the same periods.

The Company holds debt securities of companies with high credit quality and has determined that there was no material change in the credit risk of any of its debt securities. The contractual maturity dates of all the investments are less than one year.

#### 6. Accrued expenses and other current liabilities

Accrued expenses and other current liabilities consist of the following (in thousands):

	N	1arch 31, 2021	Dec	ember 31, 2020
Professional fees	\$	3,246	\$	1,948
Other research costs		3,059		2,423
Process development and manufacturing costs		2,042		2,272
Employee compensation and related benefits		1,821		7,591
Other		4,808		4,253
Total	\$	14,976	\$	18,487

#### 7. Leases

#### **Operating leases**

The Company's operating leases are as follows:

- A February 2018 lease for 38,203 square feet of office and laboratory space, which commenced in March 2018 and terminates in September 2028. The lease is subject to fixed-rate rent escalations and provided for \$6.1 million in tenant improvements and a term extension option, which was not reasonably certain of exercise.
- An October 2018 lease for laboratory space, which commenced in April 2019 and was amended in March 2020 and April 2020. The amended lease commenced in April 2020 and terminates in December 2025. The amended lease is subject to fixed-rate rent escalations and provides an option to extend the lease for two additional two-year periods through December 31, 2029, which were not reasonably certain of being exercised. Upon commencement of the March 2020 amendment, the Company recorded an operating lease right-of-use, or ROU, asset and a lease liability of \$4.2 million. Upon commencement of the April 2020 amendment, the Company recorded an operating lease ROU asset and a lease liability of \$1.8 million.
- Leases in June and July 2019 for office and laboratory space, both of which commenced in October 2019 and terminate in December 2021. The leases are subject to fixed-rate rent escalations.
- An April 2019 lease for office and laboratory space to be built, with the rent payments for the first phase expected to commence at the earliest in the second half of 2021 and the rent payments for the second phase expected to commence at the earliest in the first half of 2022. The lease will terminate 12 years from the second phase rent commencement date. The lease is subject to fixed-rate rent escalations and provides for \$23.4 million in tenant improvements and the option to extend the lease for two terms of five years each, which are not reasonably certain of exercise. The Company determined that it is the accounting owner of all tenant improvements. Upon executing the lease, the Company made a security deposit of \$11.8 million in the form of a letter of credit, which is included in restricted cash as of March 31, 2021 and December 31, 2020. Upon commencement of the first phase of this lease in October 2020, the Company recorded an operating lease ROU asset of \$66.8 million and a lease liability of \$68.8 million and a corresponding lease liability of \$23.0 million.

The following table summarizes operating lease costs as well as sublease income (in thousands):

	 Three Months Ended March 31,				
	2021 2020				
Operating lease costs	\$ 4,558	\$	1,598		
Variable lease costs	210		288		
Total	\$ 4,768	\$	1,886		

The following table summarizes the lease term and discount rate for operating leases:

	March 31, 2021	December 31, 2020
Weighted-average remaining lease term (years)	11.7	11.5
Weighted-average discount rate	7.1%	7.4%

The following table summarizes the lease costs for amounts included in the measurement of lease liabilities (in thousands):

	 Three Mor Mare	 ed
	2021	2020
Operating cash flows used for operating leases	\$ 1,685	\$ 1,525
Operating lease liabilities arising from obtaining ROU assets	23,366	4,032

At March 31, 2021, the future minimum lease payments for the Company's operating leases for each of the years ending December 31 were as follows (in thousands):

Remainder of 2021	\$ 7,943
2022	16,121
2023	17,231
2024	17,756
2025	18,228
Thereafter	133,945
Undiscounted lease payments	211,224
Less: imputed interest	(81,514)
Total operating lease liabilities	\$ 129,710

In August 2020, the Company entered into a lease agreement with Alexandria Real Estate Equities, Inc. to build a 100,000 square foot manufacturing facility in Research Triangle Park, North Carolina intended to support a broad range of clinical programs. The lease has a term of fifteen years following the commencement date and provides the Company the option to extend the lease term for two five-year terms. It is subject to fixed rate escalation increases and also provides up to \$20.0 million for reimbursement of tenant improvements. As the lease had not commenced as of March 31, 2021, the Company has not recorded an operating lease ROU asset or lease liability for this lease in the accompanying condensed consolidated balance sheets. The lease payments are subject to adjustment following the determination of the total project costs of the landlord. The initial estimate of the minimum amount of undiscounted lease payments due under this lease is \$63.9 million. The Company expects to invest up to \$83.0 million over a five-year period and anticipates that the facility will be operational by the first quarter of 2023. Further, the tabular disclosure of minimum lease payments above does not include payments due under this lease.

#### 8. Guide Acquisition

On February 23, 2021, the Company entered into the Guide Merger Agreement. Under the Guide Merger Agreement, the Company paid Guide's former stockholders and optionholders upfront consideration in an aggregate amount of \$120.0 million, excluding customary purchase price adjustments and closing costs, in shares of the Company's common stock, based upon the volume-weighted average price of the Company's stock over the ten trading day period ending on February 19, 2021. Pursuant to the Guide Merger Agreement, Beam acquired all of the issued and outstanding shares of Guide. The Company issued a total of 1,087,153 shares of its common stock valued at \$120.0 million in connection with the upfront payments to Guide's former stockholders and optionholders. The Guide transaction resulted in the acquisition of certain know-how and intellectual property assets related to Guide's proprietary *in vivo* LNP screening technology and its library of lipids and lipid nanoparticle formulations identified using the screening technology. Management determined that the acquired assets do not meet the definition of a business pursuant to ASC 805, *Business Combinations*, as substantially all of the fair value of the acquired assets is concentrated into one identifiable asset, the LNP screening technology and associated lipid library. As of the Merger Agreement Date, the asset acquired had no alternative future use and had not reached a stage of technological feasibility. As a result, all share-based and cash payment obligations have been recorded as research and development expense in the accompanying condensed consolidated statements of operations and other comprehensive loss in the amount of \$155.0 million. The total transaction price was allocated to the assets acquired and liabilities assumed on a relative fair value basis

In addition, Guide's former stockholders and optionholders will be eligible to receive up to an additional \$100.0 million in technology and \$220.0 million in product milestone payments, payable in the Company's common stock valued using the volume-weighted average price of the Company's stock over the ten-day trading period ending two trading days prior to the date on which the applicable milestone is received.

The Company determined that all future technology and product milestone payments are classified as contingent consideration liabilities under ASC 480 and therefore the Company recorded a liability for these milestones as of the Guide Merger Agreement Date at fair value of \$36.5 million. These contingent consideration liabilities will be remeasured at fair value each financial reporting period, with the resulting impact reflected in the Company's condensed consolidated statements of operations and other comprehensive loss, presented within other income (expense)

The transaction price was determined and allocated as follows (in thousands):

Transaction price	
Fair value of equity instruments issued	\$ 120,032
Technology and product contingent consideration liabilities	36,513
Transaction costs	2,531
Total transaction price	\$ 159,076
Transaction price allocated	
In-process research and development	\$ 154,953
Cash acquired	3,151
Prepaid expenses and other assets	264
Property and equipment	1,835
Assembled workforce	300
Other liabilities assumed	 (1,427)
Total transaction price	\$ 159,076

#### 9. License agreements

#### Harvard license agreement

Under the terms of the Harvard License Agreement, Harvard is entitled to receive success payments determined based upon the achievement of specified multiples of the initial weighted average value of the Company's Series A Preferred Stock at specified valuation dates. The success payments range from \$5.0 million to a maximum of \$105.0 million and have valuation multiples that range from 5 times to 40 times the initial weighted average value of the Series A Preferred Stock. Subsequent to the Company's February 2020 IPO, the amount of success payments is based on market value of Beam's common stock.

The Company is required to make success payments to Harvard during a period of time, or the Harvard Success Payment Period, which has been determined to be the later of (1) the ninth anniversary of the Harvard License Agreement or (2) the earlier of (a) the twelfth anniversary of the Harvard License Agreement and (b) the third anniversary of the first date on which a licensed product receives regulatory approval in the United States. During the Harvard Success Payment Period, the Company will perform a calculation of any amounts owed to Harvard on each rolling 90-day period, commencing one year after the Company's IPO.

The following table summarizes the Company's success payment liability for Harvard (in thousands):

	March 31, 2021	December 31, 2020	
Harvard success payment liability	\$ 36,500	\$	35,500

The following table summarizes the expense resulting from the change in the fair value of the success payment liability for Harvard (in thousands):

	Three Months Ended March 31,			
		2021		2020
Change in fair value of Harvard success payment liability	\$	1,000	\$	1,300

As of March 31, 2021, no success payments have been paid or are due to Harvard.

In May 2021, the first success payment measurement occurred and amounts due to Harvard were calculated to be \$15.0 million. The Company has elected to make the payment in shares of the Company's common stock, and thereby expects to issue shares of the Company's common stock to settle this liability in June 2021.

The annual maintenance fee under the Harvard License Agreement is recorded as research and development expense. Patent prosecution costs are recognized as expense in the period incurred. As of March 31, 2021, the Company determined that product development and regulatory approval milestones and royalties under the Harvard License Agreement were not probable and, as such, no amounts were recognized for the three months ended March 31, 2021.

#### Broad license agreement

Under the terms of the Broad License Agreement, Broad Institute is entitled to receive success payments, determined based upon the achievement of specified multiples of the initial weighted average value of Series A Preferred Stock at specified valuation dates. The success payments range from \$5.0 million to a maximum of \$105.0 million and have valuation multiples that range from 5 times to 40 times the initial weighted average value of the Series A Preferred Stock. Subsequent to the Company's February 2020 IPO, the amount of success payments is based on market value of Beam's common stock.

The Company is required to make success payments to Broad Institute during a period of time, or the Broad Success Payment Period, which has been determined to be the earliest of (1) the twelfth anniversary of the Broad License Agreement, or (2) the third anniversary of the first date on which a licensed product receives regulatory approval in the United States. During the Broad Success Payment Period, the Company will perform a calculation of any amounts owed to Broad Institute on each rolling 90-day period, commencing one year after the Company's IPO.

The following table summarizes the Company's success payment liability for Broad Institute (in thousands):

	urch 31, 2021	December 31, 2020		
Broad Institute success payment liability	\$ 36,600		35,700	

The following table summarizes the expense resulting from the change in the fair value of the success payment liability for Broad Institute (in thousands):

	 Three Months Ended March 31,			
	2021	2	2020	
Change in fair value of Broad Institute success payment liability	\$ 900	\$	1,400	

As of March 31, 2021, no success payments have been paid or are due to Broad Institute.

In May 2021, the first success payment measurement occurred and amounts due to Broad Institute were calculated to be \$15.0 million. The Company has elected to make the payment in shares of the Company's common stock, and thereby expects to issue shares of the Company's common stock to settle this liability in June 2021.

The annual maintenance fee under the Broad License Agreement is recorded as research and development expense. Patent prosecution costs are recognized as expense in the period incurred. As of March 31, 2021, the Company determined that product development and regulatory approval milestones and royalties under the Broad License Agreement were not probable and, as such, no amounts were recognized for the three months ended March 31, 2021.

#### Editas license agreement

In May 2018, the Company entered into a license agreement, or the Editas License Agreement, with Editas Medicine, Inc., or Editas. Pursuant to the Editas License Agreement, Editas granted to the Company licenses and options to acquire licenses to certain intellectual property rights owned or controlled by Editas, for specified uses.

The annual maintenance fees under the Editas License Agreement are recorded as research and development expense. Annual patent costs are expensed as incurred. In addition, the Company is required to make certain development, regulatory and commercial milestone payments to Editas upon the achievement of specified milestones. As of March 31, 2021, the triggering of these milestone payments was not probable and, as such, no amounts were recognized for the three months ended March 31, 2021.



#### Bio Palette license agreement

In March 2019, the Company entered into a license agreement with Bio Palette Co., Ltd., or Bio Palette, pursuant to which Beam received an exclusive (even as to Bio Palette), sublicensable license under certain patent rights related to base editing owned or controlled by Bio Palette to exploit products for the treatment of human disease throughout the world, but excluding products in the microbiome field in Asia (the "Bio Palette License Agreement"). In addition, the Company granted Bio Palette an exclusive (even as to Beam) license under certain patent rights related to base editing and gene editing owned or controlled by the Company to exploit products in the microbiome field in Asia. Each party to the agreement retains non-exclusive rights to develop and manufacture products in the microbiome field worldwide for the sole purpose of exploiting those products in its own territory. Each party agrees to certain coordination obligations in the microbiome field if either party determines not to exploit their rights in such field. Unless earlier terminated, the Bio Palette License Agreement will expire on a licensed product-by-licensed product and country-by-country basis upon the expiration of the applicable royalty term for each such licensed product and country.

Upon the issuance of a certain Bio Palette patent in the United States in June 2020, the Company made a milestone payment of \$2.0 million and, in July 2020, issued to Bio Palette 175,000 shares of its common stock valued at \$0.3 million. As of March 31, 2020, the Company determined that the patent was probable of issuance and recognized \$2.3 million as research and development expense for the three months ended March 31, 2020.

Management concluded that the licenses acquired from each transaction above did not meet the accounting definition of a business as inputs, but no processes or outputs were acquired with the licenses, and the licensed technology had not achieved technological feasibility. As the inputs that were acquired along with the licenses do not constitute a "business," the transactions have been accounted as asset acquisitions. As of the date of each License Agreement, the assets acquired had no alternative future use and the assets had not reached a stage of technological feasibility. As a result, all share-based and cash payment obligations have been recorded as research and development expense in the accompanying consolidated statements of operations and other comprehensive loss.

#### 10. Collaboration and license agreements

#### **Prime Medicine**

In September 2019, the Company entered into a collaboration and license agreement with Prime Medicine Inc., or Prime Medicine, to research and develop a novel gene editing technology developed by one of Beam's founders. Under the terms of the agreement, the Company granted Prime Medicine a nonexclusive license to certain of its CRISPR technology (including Cas12b), delivery technology and certain other technology controlled by Beam to develop and commercialize gene editing products for the treatment of human diseases. The Company is not currently using the intellectual property licensed from Prime Medicine in any of its current programs, but it is required to use commercially reasonable efforts to develop new product candidates using the intellectual property licensed from Prime Medicine. Additionally, each party granted to the other party certain exclusive and non-exclusive licenses to certain technology developed after the effective date of the agreement and controlled by the granting party or jointly owned by the parties. Each party has an obligation to assign rights in certain technology developed under the collaboration to the other party.

Beam had an obligation to issue \$5.0 million in shares of its common stock to Prime Medicine, and Prime Medicine had an obligation to issue 5,000,000 shares of its common stock to Beam, should Beam elect to extend the collaboration beyond one year. In September 2020, the Company elected to continue the collaboration and, in October 2020, issued 200,307 shares of the Company's common stock to Prime Medicine. The Company recognized \$5.5 million, which represented the fair value of Beam's common stock issued to Prime Medicine, as research and development expense during the year ended December 31, 2020. Additionally, in October 2020, the Company received 5,000,000 shares of Prime Medicine's common stock and recognized \$0.1 million as an offset to research and development expense for the year ended December 31, 2020.

Additionally, the Company provided immaterial interim management and startup services to Prime Medicine through March 2021.

As of March 31, 2021, the Company determined that future milestones and royalties under the agreement were not probable of recognition.

#### Verve

In April 2019, Beam entered into a collaboration and license agreement, or the Verve Agreement, with Verve Therapeutics, Inc., or Verve, to investigate gene editing strategies to modify genes associated with an increased risk of coronary diseases. Under the terms of the Verve Agreement, the Company granted Verve an exclusive license to certain base editor technology and certain delivery technology, and improvements and Verve granted Beam a non-exclusive license under certain know-how and patents controlled by Verve, an interest in joint collaboration technology and an exclusive license (except as to Verve) under certain delivery technology.

As of March 31, 2021, the Company determined that milestones and royalties under the Verve Agreement were not probable of recognition.



#### 11. Preferred and common stock

In January 2020, the Company authorized preferred stock issuable of 25,000,000 shares and increased its authorized common stock issuable to 250,000,000 shares, both with a \$0.01 par value per share.

In February 2020, the Company completed its IPO in which the Company issued and sold 12,176,471 shares of its common stock, including 1,588,235 shares pursuant to the full exercise of the underwriters' option to purchase additional shares, at a public offering price of \$17.00 per share, for aggregate gross proceeds of \$207.0 million. The Company received approximately \$188.3 million in net proceeds after deducting underwriting discounts and offering expenses payable by the Company. In connection with the IPO, all outstanding shares of the Company's preferred stock converted into 29,127,523 shares of the Company's common stock.

In October 2020, the Company issued and sold 5,750,000 shares of its common stock, including 750,000 shares pursuant to the full exercise of the underwriters' option to purchase additional shares, at a public offering price of \$23.50 per share, for aggregate gross proceeds of \$135.1 million. The Company received approximately \$126.6 million in net proceeds after deducting underwriting discounts and offering expenses payable by the Company.

In October 2020, due to the Company's election to continue the collaboration agreement between Beam and Prime Medicine, the Company issued 200,307 shares of the Company's common stock to Prime Medicine.

In January 2021, the Company issued and sold 2,795,700 shares of its common stock in a private placement at an offering price of \$93.00 per share for aggregate gross proceeds of \$260.0 million. The Company received \$252.0 million in net proceeds after deducting fees to the placement agents and offering expenses payable by the Company.

In April 2021, the Company entered into an At-The-Market ("ATM") sales agreement with Jefferies LLC ("Jefferies"), under which the Company may offer and sell, from time to time at prevailing market prices, shares of the Company's common stock having aggregate gross proceeds of up to \$300.0 million. The sales agent will be entitled to compensation at a commission rate of 3.0% of the aggregate gross sales price per share sold by it under the sales agreement. In the second quarter of 2021, the Company began to issue and sell securities under this sales agreement. The Company sold 12,607 shares of its common stock under this agreement subsequent to March 31, 2021 through May 4, 2021 for net cash proceeds of \$0.8 million, after deducting commission fees and offering expenses payable by the Company.

In May 2021, the first success payment measurements occurred and success payments to Harvard and Broad Institute were calculated to be \$15.0 million and \$15.0 million, respectively. The Company elected to make each payment in shares of the Company's common stock, and thereby expects to issue shares of the Company's common stock to settle these liabilities to Harvard and Broad Institute in June 2021.

The holders of the Company's common stock are entitled to one vote for each share of common stock. Subject to the payment in full of all preferential dividends to which the holders of the Company's preferred stock are entitled, the holders of the Company's common stock shall be entitled to receive ratably dividends out of funds legally available. In the event of any voluntary or involuntary liquidation, dissolution, or winding up of the Company, after the payment or provision for payment of all debts and liabilities of the Company and all preferential amounts to which the holders of Company's preferred stock are entitled with respect to the distribution of assets in liquidation, the holders of common stock shall be entitled to share ratably in the remaining assets of the Company available for distribution.

#### 12. Stock option and grant plan

#### Stock option and grant plan

The Beam Therapeutics Inc. 2017 Stock Option and Grant Plan adopted by the Company's board of directors in June 2017 and amended in February and May 2019, provides for the grant of qualified incentive stock options and nonqualified stock options, restricted stock awards, restricted stock units, or other awards to the Company's employees, officers, directors, advisors, and outside consultants for the issuance or purchase of shares of the Company's common stock.

In October 2019, the Company's board of directors adopted the Beam Therapeutics Inc. 2019 Equity Incentive Plan, or the 2019 Plan, and, subsequent to the IPO, all equity-based awards are granted under the 2019 Plan. The 2019 Plan provides for grant of qualified and nonqualified stock options, stock appreciation rights, restricted and unrestricted stock and stock units, performance awards, and other share-based awards to the Company's employees, officers, directors, advisors, and outside consultants. As of March 31, 2021, the Company had 9,594,873 shares reserved and 2,891,667 shares available for future issuance.

Stock-based compensation expense recorded as research and development and general and administrative expenses in the condensed consolidated statements of operations and other comprehensive loss is as follows (in thousands):

	 Three Months Ended March 31,				
	2021	2020			
Research and development	\$ 3,155	\$	1,773		
General and administrative	1,493		1,019		
Total stock-based compensation expense	\$ 4,648	\$	2,792		

#### Stock options

A summary of option activity under the Company's equity award plans:

	Number of options	Weighted average exercise price
Outstanding at December 31, 2020	5,336,441	\$ 9.70
Granted	992,659	80.89
Exercised	(199,284)	8.82
Forfeitures	(21,690)	9.06
Outstanding at March 31, 2021	6,108,126	21.30
Exercisable as of March 31, 2021	1,720,606	5.64

The weighted-average grant date fair value per share of options granted in the three months ended March 31, 2021 was \$53.59. As of March 31, 2021, there was \$79.4 million of unrecognized compensation expense related to unvested stock options, which is expected to be recognized over a weighted-average period of approximately 2.6 years.

#### **Restricted stock**

The Company issues shares of restricted common stock, including both restricted stock units and restricted stock awards. Restricted common stock issued generally vests over a period of two to four years.

The following summarizes the Company's restricted stock activity:

	Shares	av	Weighted- /erage grant date fair value
Unvested as of December 31, 2020	1,275,338	\$	10.95
Issued	511,580		80.04
Vested	(398,804)		3.30
Unvested as of March 31, 2021	1,388,114	\$	37.64

At March 31, 2021, there was approximately \$53.0 million of unrecognized stock-based compensation expense related to restricted stock that is expected to vest. These costs are expected to be recognized over a weighted-average period of approximately 2.2 years.

#### 13. Net loss per share attributable to common stockholders

As noted above, for periods in which the Company reports a net loss attributable to common stockholders, potentially dilutive securities have been excluded from the computation of diluted net loss per share as their effects would be anti-dilutive. Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at period end, from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect:

	As of March	31,
	2021	2020
Unvested restricted stock	1,388,114	2,267,940
Outstanding options to purchase common stock	6,108,126	5,663,598
Total	7,496,240	7,931,538

The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company (in thousands except share and per share amounts):

	 Three Months Ended March 31,				
	2021	2020			
Numerator:					
Net loss attributable to common stockholders	\$ (201,560)	\$	(31,735)		
Denominator:					
Weighted average number of common shares, basic and diluted	60,210,120		30,725,077		
Net loss per common share attributable to common stockholders, basic and diluted	\$ (3.35)	\$	(1.03)		

#### 14. Income taxes

During the three months ended March 31, 2021 and 2020, the Company recorded a full valuation allowance on federal and state deferred tax assets since management does not forecast the Company to be in a taxable position in the near future.

#### 15. Related party transactions

#### Founders

For the three months ended March 31, 2021 and 2020, the Company made payments of \$0.1 million and \$0.1 million, respectively, to its three founder shareholders for scientific consulting and other expenses.

#### Verve

The Company and Verve are parties to a collaboration and license agreement and have a common board member. During the three months ended March 31, 2020, the Company purchased shares of Verve series A preferred stock valued at \$0.8 million. During the three months ended March 31, 2021, the Company recorded unrealized gains of \$1.0 million on its investment in Verve preferred stock.

The Company purchased certain materials from Verve amounting to \$0.2 million, which are recorded as research and development expenses within the accompanying condensed consolidated statements of operations and other comprehensive loss, for the three months ended March 31, 2021.

#### **Prime Medicine**

The Company and Prime Medicine are parties to a collaboration and license agreement and have a common founder and several common board members.

Additionally, in September 2019, in connection with the Company's collaboration and license agreement with Prime Medicine, the Company executed a letter agreement, as amended, to provide certain interim management and startup services to Prime Medicine for up to March 2021. Prime Medicine was obligated to reimburse the Company's out-of-pocket costs incurred in connection with performing the services and, beginning in October 2020, paid the Company a \$30 thousand monthly service fee. For the three months ended March 31, 2021, the Company recognized \$0.1 million for performing such services in interest and other income (expense), net, within the accompanying consolidated statements of operations and other comprehensive loss.

#### 16. Subsequent Events

#### At-the-Market Offering

In April 2021, the Company entered into an ATM sales agreement with Jefferies, under which the Company may offer and sell, from time to time at prevailing market prices, shares of the Company's common stock having aggregate gross proceeds of up to \$300.0 million. The sales agent will be entitled to compensation at a commission rate of 3.0% of the aggregate gross sales price per share sold by it under the sales agreement. In the second quarter of 2021, the Company began to issue and sell securities under this sales agreement. The Company sold 12,607 shares of its common stock under this agreement subsequent to March 31, 2021 through May 4, 2021 for net cash proceeds of \$0.8 million, after deducting commission fees and offering expenses payable by the Company.

#### Success Payments

In May 2021, the first success payment measurements occurred and success payments to Harvard and Broad Institute were calculated to be \$15.0 million and \$15.0 million, respectively. The Company elected to make each payment in shares of the Company's common stock, and thereby expects to issue shares of the Company's common stock to Harvard and Broad Institute to settle these liabilities in June 2021.

#### 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 our condensed consolidated financial statements and the related notes to those statements included elsewhere in this Quarterly Report on Form 10-Q. In addition to historical financial information, the following discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Some of the numbers included herein have been rounded for the convenience of presentation. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of many factors, including those discussed in "Risk Factors" in Part II, Item 1A. and elsewhere in this Quarterly Report on Form 10-Q, and in the "Item 1A. Risk Factors" section of our Annual Report on Form 10-K for the fiscal year ended December 31, 2020.

#### Overview

We are a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. Our vision is to provide life-long cures to patients suffering from serious diseases. To achieve this vision, we have assembled a platform that includes a suite of gene editing and delivery technologies and are in the process of developing internal manufacturing capabilities. Our suite of gene editing technologies is anchored by our proprietary base editing technology, which potentially enables an entirely new class of precision genetic medicines that target a single base in the genome without making a double-stranded break in the DNA. This approach uses a chemical reaction designed to create precise, predictable and efficient genetic outcomes at the targeted sequence. Our novel base editors have two principal components: (i) a CRISPR protein, bound to a guide RNA, that leverages the established DNA-targeting ability of CRISPR, but modified to not cause a double-stranded break, and (ii) a base editing enzyme, such as a deaminase, which carries out the desired chemical modification of the target DNA base. We believe this design contributes to a more precise and efficient edit compared to traditional gene editing methods, which operate by creating targeted double-stranded breaks in the DNA; these breaks can result in unwanted DNA modifications. We believe that the precision of our editors will dramatically increase the impact of gene editing for a broad range of therapeutic applications.

To unlock the full potential of our base editing technology across a wide range of therapeutic applications, we are pursuing a broad suite of both clinically validated and novel delivery modalities. For a given tissue type, we use the delivery modality with the most compelling biodistribution. Our current programs are organized by delivery modality into three distinct pipelines: (1) electroporation for efficient delivery to blood cells and immune cells *ex vivo*; (2) lipid nanoparticles, or LNPs, for non-viral *in vivo* delivery to the liver and potentially other organs in the future; and (3) adeno-associated viral vectors, or AAV, for *in vivo* viral delivery to the eye and central nervous system, or CNS.

The elegance of the base editing approach combined with a tissue specific delivery modality provides the basis for a targeted efficient, precise, and highly versatile gene editing system, capable of gene correction, gene silencing/gene activation, and or multiplex editing of several genes simultaneously. We are currently advancing a broad, diversified portfolio of base editing programs against distinct editing targets, utilizing the full range of our development capabilities. We believe the flexibility and versatility of our base editors may lead to broad therapeutic applicability and transformational potential for the field of precision genetic medicines.

We believe that building an integrated platform combining our gene editing capabilities with advanced delivery and manufacturing capabilities will give us the maximum flexibility to develop our own sustainable portfolio and to create a hub for partnering with other companies to unlock the full potential of precision genetic medicine across all possible applications.

#### Ex vivo electroporation for hematology: Sickle cell disease and beta-thalassemia

We are using base editing to pursue the development of two complementary approaches to treating sickle cell disease, a severe inherited blood disease caused by a single point mutation, E6V, in the beta globin gene (BEAM-101 and BEAM-102), and one approach to treat beta-thalassemia, another inherited blood disorder characterized by severe anemia caused by reduced production of functional hemoglobin due to insufficient expression of the beta globin protein (BEAM-101).

#### BEAM-101: Recreating naturally-occurring protective mutations to activate fetal hemoglobin

Our development candidate, BEAM-101, reproduces single base changes seen in individuals with Hereditary Persistence of Fetal Hemoglobin, or HPFH, to potentially protect them from the effects of mutations causing sickle cell disease or beta thalassemia.

We have achieved proof-of-concept *in vivo* with long-term engraftment of base edited human CD34 cells in mice for BEAM-101. Persistence of engraftment and high levels of editing have been confirmed in several preclinical studies, including in studies using material generated at a clinically relevant scale. Following conversations with regulators and supported by our off-target biology assays, we initiated IND-enabling studies in 2020 and expect to file an IND for BEAM-101 during the second half of 2021.



#### BEAM-102: Direct correction of the sickle cell mutation

Our second base editing approach that we are developing for sickle cell disease, BEAM-102, is a direct correction of the causative sickle mutation at position 6 of the beta globin gene. This approach has been enabled by our inlaid base editors, or IBEs, which are architectural variants of base editors that have attributes of enhanced specificity and altered activity windows relative to foundational base editors. Our IBEs expand the breadth of potential base editing targets by extending the range of editing windows that can be created for any given CRISPR protein used to target the DNA. By inserting the deaminase into the CRISPR protein at various strategic positions, thereby repositioning the deaminase's editing window, IBEs enable editing outside the traditional editing window. BEAM-102 directly corrects the causative mutation in sickle cell disease by recreating a naturally-occurring normal human hemoglobin variant, HbG-Makassar. By making a single A-to-G edit, we have demonstrated in primary human CD34+ cells isolated from sickle cell disease patients the ability to create the naturally occurring Makassar variant of hemoglobin. This variant, which was identified in humans and first published in 1970, has the same function as the wild-type variant and does not cause sickle cell disease. Distinct from other approaches, cells that are successfully edited in this way are fully corrected, no longer containing the sickle protein.

During the second quarter of 2020, we published preclinical data on BEAM-102 demonstrating that our adenine base editors, or ABEs, can efficiently convert the causative Hemoglobin S, or HbS, point mutation, to HbG-Makassar, with high efficiency (more than 80%). In this preclinical study, the Makassar variant does not cause hemoglobin to polymerize and red blood cells to sickle and, therefore, edited cells are cured through elimination of the disease-causing protein. The results from this study confirmed the ability of the Makassar variant to protect cells from sickling, even in the context of mono-allelic editing (with one sickle allele and one corrected allele). We plan to initiate IND-enabling studies for BEAM-102 during 2021.

#### Ex vivo electroporation for multiplex editing: CAR-T cell therapies

The initial indications that we plan to target with our chimeric antigen receptor T cell, or CAR-T, product candidates are relapsed, refractory T-cell acute lymphoblastic leukemia, or T-ALL, a severe disease affecting children and adults, and Acute Myeloid Leukemia, or AML. We believe that our approach has the potential to produce higher response rates and deeper remissions than existing approaches. Our proof-of-concept pre-clinical experiments have demonstrated the ability of base editors to efficiently modify up to 8 genomic loci simultaneously in primary human T cells with efficiencies ranging from 85-95% as measured by flow cytometry of target protein knockdown. Importantly, these results were achieved without the generation of chromosomal rearrangements, as detected by sensitive methods such as UDiTaSTM or G-banded Karyotyping and with no loss of cell viability from editing. Our proof-of-concept experiments have also demonstrated robust T cell killing of target tumor cells both *in vitro* and *in vivo*.

#### BEAM-201: Universal CD7-targeting CAR-T cells

BEAM-201 is our potent and specific anti-CD7, multiplex edited, allogeneic CAR-T development candidate for the treatment of relapsed/refractory T-ALL. BEAM-201 is produced using a Good Manufacturing Practice, or cGMP, compliant, clinical-scale process in which T-cells derived from healthy donors are simultaneously base edited at four genomic loci, then transduced with a lentivirus coding for an anti-CD7 CAR. The resulting cells are universally-compatible, allogeneic ("off the shelf") CD7-targeting CAR-T cells, resistant to both fratricide and immunosuppression. To our knowledge, Beam-201 is the first cell therapy featuring four simultaneous edits. We plan to initiate IND-enabling studies for BEAM-201 during 2021.

#### Non-viral delivery for liver diseases: Alpha-1 antitrypsin deficiency and glycogen storage disorder 1a

We are currently using a variety of cationic lipids from various sources to advance our programs for genetic liver diseases, which include Alpha-1 Antitrypsin Deficiency, or Alpha-1, and Glycogen Storage Disease Type IA, or GSDIa (also known as Von Gierke disease).

Alpha-1 is a severe inherited genetic disorder that can cause progressive lung and liver disease. The most severe form of Alpha-1 arises when a patient has a point mutation in both copies of the SERPINA1 gene at amino acid 342 position (E342K, also known as the PiZ mutation or the "Z" allele). With the high efficiency and precision of our base editors, we aim to utilize our ABEs to enable the programmable conversion of A-to-T and G-to-C base pairs and precisely correct the E342K point mutation back to the wild type sequence.

GSDIa is an inborn disorder of glucose metabolism caused by mutations in the G6PC gene, which results in low blood glucose levels that can be fatal if patients do not adhere to a strict regimen of slow-release forms of glucose, administered every one to four hours (including overnight). Our approach to treating patients with GSDIa is to apply base editing via LNP delivery to repair the two most prevalent mutations that cause the disease, R83C and Q347X.

We continue to advance our liver disease programs. In 2020, we showed the ability to directly correct the mutation causing Alpha-1, providing both *in vitro* and *in vivo* preclinical proof-of-concept for base editing to correct this disease. We have also achieved editing levels *in vivo*, in preclinical models, for the correction of the two most prevalent mutations causing GSDIa, which could be clinically relevant if reproduced in humans.



An important next step for our liver disease programs is finalizing our LNP formulation, and we are making progress on developing a formulation using proof-of-concept targets. To date, with this formulation, we have shown high levels of editing in mice at doses consistent with clinical use. In addition, in May 2021, we announced initial data from our evaluation of various LNP formulations and mRNA production processes using an mRNA-encoding ABE and guide RNA to target the ALAS1 gene, a surrogate payload for genetic liver diseases. The data showed improved *in vivo* editing in the livers of non-human primates from less than 10% initially to 52% at a total RNA dose of 1.5 mg/kg. The data also demonstrated that these formulations were well tolerated by non-human primates at 1.5 mg/kg with mild and transient liver enzyme elevations, and showed promising interim stability, maintaining potency after 8 weeks at -20°C. We are continuing to work on optimizing our LNP formulations and believe we are on track to nominate our first development candidate from our liver portfolio in the second half of 2021.

#### Viral delivery for ocular and CNS disorders: Stargardt disease

We are currently evaluating AAV technology to correct one of the most prevalent mutations in the ABCA4 gene causing Stargardt disease, a progressive macular degeneration. This mutation is known as the G1961E point mutation.

Our base editing approach is to repair the G1961E point mutation in the ABCA4 gene. Disease modeling using tiny spot stimuli, or light stimuli through holes that are equivalent in size to a single photoreceptor cell, suggests that only 12%-20% of these cells are sufficient to preserve vision. We anticipate, therefore, that editing percentages in the range of 12%-20% of these cells would be disease-modifying, since each edited cell will be fully corrected and protected from the biochemical defect.

We have identified a base editor that is able to edit approximately 45% of the alleles in recombinant cells carrying the human mutated sequence. Given that the base editor is larger than the packaging capacity of a single AAV, we use a split AAV system that delivers the base editor via two AAV vectors. Once inside the cell, the two halves of the editor are recombined to create a functional base editor. In a human retinal pigment epithelial cell line (ARPE-19 cells) in which we have knocked in the ABCA4 G1961E point mutation, we have demonstrated the precise correction of approximately 75% of the disease alleles at 5 weeks after dual infection with the split AAV system.

#### **Delivery of genetic medicines**

To complement our next-generation gene editing technologies, we are also making significant investments in a broad suite of delivery technologies to deliver our gene editing payloads to the right cells to enable potentially curative therapy. These delivery technologies include *ex vivo* electroporation, nonviral vectors such as LNPs, and viral vectors such as AAVs. In our pipeline, we have initially focused on applications of these technologies that are clinically-validated, such as *ex vivo* editing of blood stem cells or LNP delivery to the liver. Longer term, we are also investing in more innovative delivery options, such as LNPs that could target other organs beyond the liver, or novel viral vectors beyond AAV. We have also developed critical enabling capabilities such as mRNA manufacturing and cell processing for autologous and allogeneic cell therapy.

Consistent with this approach, our recent acquisition of Guide Therapeutics, Inc., or Guide, expands our ability to explore new tissues and disease indications with our editing technologies. Guide's proprietary screening technology, which utilizes DNA barcodes to enable high throughput *in vivo* LNP screening, provides us with access to an existing broad library of lipids and lipid formulations, and the ability to generate additional novel LNPs that we believe could accelerate novel nonviral delivery of gene editing payloads to tissues beyond the liver.

#### Collaborations

We believe our base editing technology has potential across a broad array of genetic diseases. To fully realize this potential, we have established and will continue to seek out innovative collaborations, licenses, and strategic alliances with pioneering companies and with leading academic and research institutions. Additionally, we have and will continue to pursue relationships that potentially allow us to accelerate our preclinical research and development efforts. These relationships will allow us to aggressively pursue our vision of maximizing the potential of base editing to provide life-long cures for patients suffering from serious diseases.

#### Ex vivo electroporation for hematologic diseases and oncology

#### Boston Children's Hospital

In July 2020, we formed a strategic alliance with Boston Children's Hospital, or Boston Children's. Under the terms of the agreement, we will sponsor research programs at Boston Children's to facilitate development of disease-specific therapies using our proprietary base editing technology. Boston Children's will also serve as a clinical site to advance bench-to-bedside translation of our pipeline across certain therapeutic areas of interest, including programs in sickle cell disease and pediatric leukemias and exploration of new programs targeting other diseases.

#### **Magenta** Therapeutics

In June 2020, we announced a non-exclusive research and clinical collaboration agreement with Magenta Therapeutics Inc., or Magenta, to evaluate the potential utility of MGTA-117, Magenta's novel targeted antibody-drug conjugate for conditioning of patients with sickle cell disease and beta-thalassemia receiving our base editing therapies. Conditioning is a critical component necessary to prepare a patient's body to receive the edited cells, which carry the corrected gene and must engraft in the patient's bone marrow in order to be effective. Today's conditioning regimens rely on nonspecific chemotherapy or radiation, which are associated with significant toxicities. MGTA-117 is designed to precisely target only hematopoietic stem and progenitor cells, to spare immune cells, and has shown high selectivity, potent efficacy, wide safety margins and broad tolerability in non-human primate models. MGTA-117 may be capable of clearing space in bone marrow to support long-term engraftment and rapid recovery in patients. Combining the precision of our base editing technology with the more targeted conditioning regimen enabled by MGTA-117 has the potential to further improve therapeutic outcomes for patients suffering from these severe diseases. We will be responsible for clinical trial costs related to development of our base editors when combined with MGTA-117, while Magenta will continue to be responsible for all other development costs of MGTA-117.

#### Non-Viral delivery for liver diseases

#### Verve Therapeutics

In April 2019, we entered into a collaboration and license agreement with Verve Therapeutics Inc., or Verve, a company focused on developing genetic medicines to safely edit the genome of adults to permanently lower LDL cholesterol and triglyceride levels and thereby treat coronary heart disease. This collaboration allows us to more fully realize the potential of base editing in treating cardiovascular diseases, an area outside of our core focus where the Verve team has significant, world-class expertise. Under the terms of the agreement, Verve received exclusive access to our base editing technology, gene editing, and delivery technologies for human therapeutic applications against certain cardiovascular targets. In exchange, we received 2,556,322 shares of Verve common stock. Additionally, we will receive milestone payments for certain clinical and regulatory events and we retain the option, after the completion of Phase 1 studies, to participate in future development and commercialization, and share 50 percent of U.S. profits and losses, for any product directed against these targets. Verve granted to us a non-exclusive license under know-how and patents controlled by Verve, and an interest in joint collaboration technology. Either party may owe the other party other milestone payments for certain clinical and regulatory events related to the delivery technology products. Royalty payments may become due by either party to the other based on the net sales of any commercialized delivery technology products under the agreement.

In January 2021, Verve announced it had selected VERVE-101 as its lead product to be developed initially for the treatment of heterozygous familial hypercholesterolemia, or HeFH, a potentially fatal genetic heart disease. Individuals with HeFH have a genetic mutation causing extremely high LDL-C levels in the blood. Over time, high LDL-C builds up in the heart's arteries, resulting in reduced blood flow or blockage, and ultimately heart attack or stroke. Inactivation of the proprotein convertase subtilisin/kexin type 9, or PCSK9, gene has been shown to up-regulate LDL receptor expression, which leads to lower LDL-C levels. By making a single A-to-G change in the DNA genetic sequence of PCSK9, VERVE-101 aims to inactivate the target gene.

In January 2021, Verve also reported additional preclinical proof-of-concept data in non-human primates that demonstrated the successful use of ABEs to turn off PCSK9. Utilizing ABE technology licensed from us and an optimized guide RNA packaged in an engineered lipid nanoparticle, Verve announced data demonstrating that *in vivo* base editing of the PCSK9 gene in the liver of non-human primates resulted in durable and consistent lowering of blood LDL-C and blood PCSK9 protein levels following a single course of treatment. In its studies, Verve reported that a single intravenous infusion achieved a 59% reduction in blood LDL-C at two weeks, which was maintained at six months post treatment, and that LDL-C reduction over this time period averaged 61%. Verve also disclosed that during this same six-month time period, the average blood PCSK9 protein level was reduced by 89%. Verve further reported that the treatment was well tolerated with no adverse events reported during the study and that in studies of primary human hepatocytes, clear evidence of on-target editing was observed with no evidence of off-target editing.

Per the terms of our agreement with Verve, we can exercise our right to participate in the future development and commercialization of any programs at the completion of Phase 1 studies.

#### Viral delivery for ophthalmology and CNS diseases

#### Institute of Molecular and Clinical Ophthalmology Basel

In July 2020, we announced a research collaboration with the Institute of Molecular and Clinical Ophthalmology Basel, or IOB. Founded in 2018 by a consortium that includes Novartis, the University Hospital of Basel and the University of Basel, IOB is a leader in basic and translational research aimed at treating impaired vision and blindness. Clinical scientists at IOB have also helped to develop better ways to measure how vision is impacted by Stargardt disease. Additionally, researchers at IOB have developed living models of the retina, known as organoids, which can be used to test novel therapies. Under the terms of the agreement, the companies will leverage IOB's unique expertise in the field of ophthalmology along with our novel base editing technology to advance programs directed to the treatment of certain ocular diseases, including Stargardt disease.



#### Manufacturing

To realize the full potential of base editors as a new class of medicines and to enable our parallel investment strategy in multiple delivery modalities, we are building customized and integrated capabilities across discovery, manufacturing, and preclinical and clinical development. Due to the critical importance of high-quality manufacturing and control of production timing and know-how, we have taken steps toward establishing our own manufacturing facility, which will provide us the flexibility to manufacture numerous different drug product modalities. We believe this investment will maximize the value of our portfolio and capabilities, the probability of technical success of our programs, and the speed at which we can provide life-long cures to patients.

In August 2020, we entered into a lease agreement with Alexandria Real Estate Equities, Inc. to build a 100,000 square foot current cGMP compliant manufacturing facility in Research Triangle Park, North Carolina intended to support a broad range of clinical programs. We will invest up to \$83.0 million over a five-year period and anticipate that the facility will be operational by the first quarter of 2023. The project will be facilitated, in part, by a Job Development Investment Grant approved by the North Carolina Economic Investment Committee, which authorizes potential reimbursements based on new tax revenues generated through the project. The facility will be designed to support manufacturing for our *ex vivo* cell therapy programs in hematology and *oncology* and *in vivo* non-viral delivery programs for liver diseases, with flexibility to support manufacturing of our viral delivery programs, and ultimately, scale-up to support potential commercial supply.

For our initial waves of clinical programs, we will use contract manufacturing organizations, or CMOs, with relevant manufacturing experience in genetic medicines.

#### Acquisitions

In February 2021, we entered into an Agreement and Plan of Merger, or the Guide Merger Agreement, to acquire Guide Therapeutics, Inc., or Guide. Pursuant to the Guide Merger Agreement, we paid Guide's former stockholders and optionholders upfront consideration in an aggregate amount of \$120.0 million, excluding customary purchase price adjustments, in shares of our common stock, based upon the volume-weighted average price of the common stock over the ten trading day period ending on February 19, 2021. In addition, Guide's former stockholders and optionholders will be eligible to receive up to an additional \$100.0 million in technology milestone payments and \$220.0 million in product milestone payments, payable in our common stock.

#### COVID-19

With the ongoing concern related to the COVID-19 pandemic in the year ended December 31, 2020 and the three months ended March 31, 2021, we have maintained and expanded the business continuity plans to address and mitigate the impact of the COVID-19 pandemic on our business. In March 2020, to protect the health of our employees, and their families and communities, we restricted access to our offices to personnel who performed critical activities that must be completed on-site, limited the number of such personnel that can be present at our facilities at any one time, and requested that most of our employees work remotely. In May 2020, as certain states eased restrictions, we established new protocols to better allow our full laboratory staff access to our facilities. These protocols included several shifts working over a seven-day week protocol. We expect to continue incurring additional costs to ensure we adhere to the guidelines instituted by the CDC and to provide a safe working environment to our onsite employees.

The extent to which the COVID-19 pandemic impacts our business, our corporate development objectives, results of operations and financial condition, including and the value of and market for our common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the duration, scope and severity of the pandemic, the duration and extent of travel restrictions and social distancing in the United States and other countries, business closures and business disruptions, the effectiveness of actions taken in the United States and other countries to contain and treat the disease, periodic spikes in infection rates, new strains of the virus that cause outbreaks of COVID-19, and the broad availability of effective vaccines. Disruptions to the global economy, disruption of global healthcare systems, and other significant impacts of the COVID-19 pandemic could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

While the COVID-19 pandemic did not significantly impact our business or results of operations during the three months ended March 31, 2021, the length and extent of the pandemic, its consequences, and containment efforts will determine the future impact on our operations and financial condition.

#### Critical accounting policies and significant judgements

Our critical accounting policies are those policies which require the most significant judgments and estimates in the preparation of our condensed consolidated financial statements. We have determined that our most critical accounting policies are those relating to stock-based compensation, variable interest entities, fair value measurements, and leases. There have been no significant changes to our existing critical accounting policies and significant accounting policies discussed in our Annual Report on Form 10-K for the year ended December 31, 2020 except as discussed below.

#### Asset Acquisitions

In 2018, we adopted ASU 2017-01, *Business Combinations*, or ASU 2017-01, which clarified the definition of a business. We measure and recognize asset acquisitions that are not deemed to be business combinations based on the cost to acquire the assets, which includes transaction costs, and the consideration is allocated to the items acquired based on a relative fair value methodology. Goodwill is not recognized in asset acquisitions. In an asset acquisition, the cost allocated to acquire in-process research and development with no alternative future use is charged to research and development expense at the acquisition date.

At the time of acquisition, we determine if a transaction should be accounted for as a business combination or acquisition of assets.

#### **Contingent Consideration Liabilities**

We may be required to make milestone payments to the former stockholders and optionholders of Guide in the form of our common stock based on the achievement of certain product and technology milestones. The payments are accounted for under ASC 480, *Distinguishing Liabilities from Equity*. These contingent consideration liabilities are carried at fair value which was estimated by applying a probability-based model, which utilized inputs that were unobservable in the market. The estimated fair value of contingent consideration liabilities, initially measured and recorded on the acquisition date, are considered to be a Level 3 instrument and are reviewed quarterly, or whenever events or circumstances occur that indicate a change in fair value. The contingent consideration liabilities are recorded at fair value at the end of each reporting period with changes in estimated fair values recorded in other income (expense) in the condensed consolidated statements of operations.

The estimated fair value is determined based on probability adjusted discounted cash flow models that include significant estimates and assumptions pertaining to technology and product development. Significant changes in any of the probabilities of success would result in a significantly higher or lower fair value measurement. Significant changes in the probabilities as to the periods in which milestones will be achieved would result in a significantly lower or higher fair value measurement.

#### **Financial operations overview**

#### General

We were incorporated on January 25, 2017 and commenced operations shortly thereafter. Since our inception, we have devoted substantially all of our resources to building our base editing platform and advancing development of our portfolio of programs, establishing and protecting our intellectual property, conducting research and development activities, organizing and staffing our company, business planning, raising capital and providing general and administrative support for these operations. To date, we have financed our operations primarily through the sales of our redeemable convertible preferred stock and proceeds from our Initial Public Offering, or IPO, our follow-on offering, and private placement.

We are a development stage company, and all of our programs are at a preclinical stage of development. To date, we have not generated any revenue from product sales and do not expect to generate revenue from the sale of products for the foreseeable future. Since inception we have incurred significant operating losses. Our net losses for the three months ended March 31, 2021 and 2020 were \$201.6 million and \$30.5 million, respectively. As of March 31, 2021, we had an accumulated deficit of \$599.2 million. We expect to continue to incur significant expenses and increasing operating losses in connection with ongoing development activities related to our portfolio of programs as we continue our preclinical development of product candidates; advance these product candidates toward clinical development; build and operate our current Good Manufacturing Practice, or cGMP, facility in North Carolina, further develop our base editing platform; continue to make investments in delivery technology for our base editors, including in connection with our recent acquisition of Guide; conduct research activities as we seek to discover and develop additional product candidates; maintain, expand, enforce, defend and protect our intellectual property portfolio; and continue to hire research and development, clinical and commercial personnel. In addition, we expect to continue to incur additional costs associated with operating as a public company and implementing controls over financing reporting.

As a result of these anticipated expenditures, we will need additional financing to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. We may be unable to raise additional funds or enter into such other agreements when needed on favorable terms or at all. Our inability to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We can give no assurance that we will be able to secure such additional sources of funds to support our operations, or, if such funds are available to us, that such additional funding will be sufficient to meet our needs.



#### Research and development expenses

Research and development expenses consist of costs incurred in performing research and development activities, which include:

- Expenses incurred in connection with investments in delivery technology for our base editors, including as a result of our acquisition of Guide;
- the cost to obtain licenses to intellectual property, such as those with Harvard University, or Harvard, Broad Institute of MIT and Harvard, or Broad Institute, and Editas Medicine, Inc, or Editas, and related future payments should certain success, development and regulatory milestones be achieved;
- personnel-related expenses, including salaries, bonuses, benefits and stock-based compensation for employees engaged in research and development functions;
- expenses incurred in connection with the discovery and preclinical development of our research programs, including under agreements with third parties, such as consultants, contractors and contract research organizations;
- expenses incurred in connection with the building of our base editing platform;
- the cost of manufacturing for use in our preclinical studies and future clinical trials;
- laboratory supplies and research materials; and
- facilities, depreciation and other expenses which include direct and allocated expenses.

We expense research and development costs as incurred. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the benefits are consumed.

In the early phases of development, our research and development costs are often devoted to product platform and proof-of-concept studies that are not necessarily allocable to a specific target.

We expect that our research and development expenses will increase substantially in connection with our planned preclinical and future clinical development activities.

#### General and administrative expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in our administrative functions. General and administrative expenses also include allocated facility related expenses and other operating costs.

We anticipate that our general and administrative expenses will increase in the future to support increased research and development activities. We also expect to continue to incur increased costs associated with being a public company and implementing controls over financial reporting, including costs of accounting, audit, legal, regulatory and tax-related services associated with maintaining compliance with Nasdaq and SEC requirements, director and officer insurance costs, and investor and public relations costs.

#### Other income and expenses

Other income and expenses consist of the following items:

- *Change in fair value of derivative liabilities* consists of remeasurement gains or losses associated with changes in success payment liabilities associated with our license agreement with Harvard, dated as of June 27, 2017, as amended, or the Harvard License Agreement, and the license agreement between Blink and Broad Institute, as amended, dated as of May 9, 2018, or the Broad License Agreement.
- Change in fair value of contingent consideration liabilities consists of remeasurement gains or losses associated with changes in the technology and product contingent consideration liabilities as part of the Guide Merger.
- *Interest and other income (expense)*, net consists primarily of interest income as well as interest expense related to our equipment financings, and mark-to-market adjustments related to our investments in equity securities.



#### **Results of operations**

### Comparison of the three months ended March 31, 2021 and 2020

The following table summarizes our results of operations (in thousands):

	Three Months Ended March 31,					
		2021 202		2020	0 Char	
License revenue	\$	6	\$	6	\$	—
Operating expenses:						
Research and development		190,106		21,549		168,557
General and administrative		10,273		6,812		3,461
Total operating expenses		200,379		28,361		172,018
Loss from operations		(200,373)		(28,355)		(172,018)
Other income (expense):						
Change in fair value of derivative liabilities		(1,900)		(2,700)		800
Change in fair value of contingent consideration liabilities		(305)		—		(305)
Interest and other income, net		1,018		597		421
Total other income (expense)		(1,187)		(2,103)		916
Net loss	\$	(201,560)	\$	(30,458)	\$	(171,102)

#### License revenue

License revenue was approximately \$6.0 thousand for the three months ended March 31, 2021 and March 31, 2020. License revenue represents Verve license revenue recorded under the Collaboration and License Agreement executed in April 2019.

#### Research and development expenses

Research and development expenses were \$190.1 million and \$21.5 million for the three months ended March 31, 2021 and 2020, respectively. The increase of \$168.6 million was primarily due to the following:

- \$155.0 million of expense related to in-process research and development asset acquired from Guide as there was determined to be no alternative future use.
- An increase of \$7.2 million in outsourced services and lab supplies, driven primarily by external research services such as CMOs, IND studies
  and sponsored research agreements.
- Increases of \$3.0 million in personnel-related costs and \$3.3 million in facility-related costs, including depreciation. These increases were due to
  the growth in the number of research and development employees from 110 at March 31, 2020 to 182 at March 31, 2021, and their related
  activities, as well as the expense allocated to research and development related to our leased facilities.
- An increase of \$1.4 million in stock compensation from additional stock option awards due to the increase in the number of research and development employees as well as an increase in the value of our common stock.
- An increase of \$1.1 million in other expenses, primarily related to an increase in research and development specific software costs.
- A decrease of \$2.3 million in milestone and license expenses. In March 2020, we recorded a \$2.3 million milestone liability as the issuance of a certain Bio Palette patent in the United States became probable.

Research and development expenses will continue to increase as we continue our current research programs, initiate new research programs, continue our preclinical development of product candidates and conduct future clinical trials for any of our product candidates.

#### General and administrative expenses

General and administrative expenses were \$10.3 million and \$6.8 million for the three months ended March 31, 2021 and 2020, respectively. The increase of \$3.5 million was primarily due to the following:

- Increases of \$1.2 million in personnel related costs and \$0.5 million in other (primarily information technology and facility related) costs due to an increase in general and administrative employees from 24 employees as of March 31, 2020 to 34 employees as of March 31, 2021.
- An increase of \$0.6 million in insurance costs due to increased directors and officers insurance costs as a result of our being a public company in February 2020.
- Increases of \$0.5 million in corporate legal and \$0.3 million in intellectual property costs due to costs related to our acquisition of Guide.
- An increase of \$0.5 million increase in stock-based compensation due to an increase in the number of general and administrative employees, as well as an increase in the value of our common stock.

#### Change in fair value of derivative liabilities

During the three months ended March 31, 2021, we recorded \$1.9 million of expense related to the change in fair value of success payment liabilities as compared to \$2.7 million expense for the three months ended March 31, 2020. The success payment obligations are still outstanding as of March 31, 2021 and will continue to be revalued at each reporting period. The first success payment will occur in June 2021.

#### Change in contingent consideration liabilities

During the three months ended March 31, 2021, we recorded \$0.3 million of expense related to the change in fair value of the Guide technology and product contingent consideration liabilities.

#### Interest and other income (expense), net

The increase in interest and other income (expense), net was primarily due to an increase in the fair value of our corporate equity securities, which are accounted for as investments in equity securities, offset by a decrease in interest income driven by decreased market rates.

#### Liquidity and capital resources

Since our inception in January 2017, we have incurred significant operating losses. We expect to incur significant expenses and operating losses for the foreseeable future as we advance the preclinical and, if successful, the clinical development of our programs. In February 2020, we completed our IPO in which we issued and sold 12,176,471 shares of our common stock, including 1,588,235 shares of common stock sold pursuant to the underwriters' full exercise of their option to purchase additional shares, at a public offering price of \$17.00 per share. We received net proceeds from our IPO of \$188.3 million, after deducting underwriting discounts and offering expenses payable by us. In October 2020, we issued and sold 5,750,000 shares of our common stock, including 750,000 shares pursuant to the full exercise of the underwriters' option to purchase additional shares, at a public offering price of \$23.50 per share, for aggregate gross proceeds of \$135.1 million. We received approximately \$126.6 million in net proceeds after deducting applicable underwriting discounts and offering expenses. In January 2021, we issued and sold 2,795,700 shares of our common stock in a private placement at an offering price of \$93.00 per share for aggregate gross proceeds of \$260.0 million. We received \$252.0 million in net proceeds after deducting offering expenses payable by us. To date, we have funded our operations primarily through equity offerings. As of March 31, 2021, we had \$503.5 million in cash, cash equivalents, and marketable securities.

In April 2021, we entered into an At-The-Market ("ATM") sales agreement with Jefferies LLC ("Jefferies"), under which we may offer and sell, from time to time at prevailing market prices, shares of our common stock having aggregate gross proceeds of up to \$300.0 million. The sales agent will be entitled to compensation at a commission rate of 3.0% of the aggregate gross sales price per share sold by it under the sales agreement. In the second quarter of 2021, we began to issue and sell securities under this sales agreement. We sold 12,607 shares of our common stock under this agreement subsequent to March 31, 2021 through May 4, 2021 for net cash proceeds of \$0.8 million, after deducting commission fees and offering expenses payable by the Company.

We are required to make success payments to Harvard and Broad Institute based on increases in the per share fair market value of our Series A-1 Preferred Stock and Series A-2 Preferred Stock or, subsequent to our IPO, our common stock. In May 2021, the first success payment measurements occurred and success payments to Harvard and Broad Institute were calculated to be \$15.0 million and \$15.0 million, respectively. We elected to make each payment in shares of our common stock, and thereby expect to issue shares of our common stock to settle these liabilities to Harvard and Broad Institute in June 2021.



To date, we have not generated any revenue from product sales and do not expect to generate revenue from the sale of products for the foreseeable future. We anticipate the need for additional capital in order to continue to fund our research and development, including our plans for preclinical and clinical trials, building, maintaining and operating a commercial-scale cGMP manufacturing facility, and new product development, as well as to fund general operations. As and if necessary, we will seek to raise these additional funds through various potential sources, such as equity and debt financings or through corporate collaboration and license agreements. Especially in light of the COVID-19 pandemic, we can give no assurances that we will be able to secure such additional sources of funds to support our operations, or, if such funds are available to us, that such additional financing will be sufficient to meet our needs. For a more detailed discussion of risks related to COVID-19, please see Part II, Item 1A, *Risk Factors—Risks related to our relationships with third parties*, included in our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the SEC on March 15, 2021.

#### Cash flows

The following table summarizes our sources and uses of cash (in thousands):

	Three Months Ended March 31,				
		2021		2020	
Net cash used in operating activities	\$	(38,578)	\$	(27,393)	
Net cash used in investing activities		(279,626)		(76,792)	
Net cash provided by financing activities		253,274		193,014	
Net change in cash, cash equivalents and restricted cash	\$	(64,930)	\$	88,829	

#### **Operating** activities

Net cash used in operating activities for the three months ended March 31, 2021 was \$38.6 million, consisting primarily of our net loss of \$201.6 million, a decrease in accrued expenses and other liabilities of \$4.5 million, an increase in prepaid expenses and other current assets of \$2.4 million, a decrease in accounts payable of \$0.7 million and other non-cash items of \$1.0 million; offset by a change in operating lease liabilities of \$6.1 million, and noncash expenses consisting primarily of in-process research and development expense of \$155.0 million related to our acquisition of Guide, stock-based compensation expense of \$4.6 million, change in operating lease ROU assets of \$2.4 million, change in fair value of derivative liabilities of \$1.9 million, depreciation and amortization expense of \$1.4 million and change in contingent consideration liabilities of \$0.3 million.

Net cash used in operating activities for the three months ended March 31, 2020 was \$27.4 million, consisting primarily of our net loss of \$30.5 million, an increase in prepaid expenses and other current assets of \$3.6 million, a decrease in operating lease liabilities of \$0.9 million, and decreases in accounts payable and accrued expenses of \$0.1 million, offset by noncash charges consisting primarily of stock-based compensation expense of \$2.8 million, change in fair value of derivative liabilities of \$2.7 million, depreciation expense of \$1.1 million, and change in operating lease ROU assets of \$1.0 million.

#### Investing activities

For the three months ended March 31, 2021, cash used in investing activities was primarily the net purchases of marketable securities of \$268.8 million, and purchases of property and equipment of \$11.5 million. We also received \$0.6 million in cash from our acquisition of Guide, after the payment of acquisition costs.

For the three months ended March 31, 2020, cash used in investing activities was primarily the net purchases of marketable securities of \$73.0 million, and purchases of property and equipment of \$3.0 million.

#### Financing activities

Net cash provided by financing activities for the three months ended March 31, 2021 consisted primarily of proceeds from our private placement offering of \$260.0 million and proceeds from the exercise of stock options of \$1.8 million, offset in part by the payment of equity offering costs of \$8.0 million and repayments of equipment financing liabilities of \$0.5 million.

Net cash provided by financing activities for the three months ended March 31, 2020 consisted primarily of proceeds from our IPO of \$192.5 million, net of underwriting discounts, and proceeds of \$1.6 million from equipment financing; offset by the payment of equity offering costs of \$1.0 million, and repayments of equipment financing liabilities of \$0.3 million.

#### Funding requirements

Our operating expenses are expected to increase substantially as we continue to advance our portfolio of programs.

Specifically, our expenses will increase if and as we:

- continue our current research programs and our preclinical development of product candidates from our current research programs;
- seek to identify additional research programs and additional product candidates;



- initiate preclinical testing and clinical trials for any product candidates we identify and develop;
- maintain, expand, enforce, defend, and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- seek marketing approvals for any of our product candidates that successfully complete clinical trials;
- establish a sales, marketing, and distribution infrastructure to commercialize any medicines for which we may obtain marketing approval;
- further develop our base editing platform;
- further develop delivery technology for our base editors, resulting from our acquisition of Guide;
- continue to hire additional personnel including research and development, clinical and commercial personnel;
- add operational, financial, and management information systems and personnel, including personnel to support our product development;
- acquire or in-license products, intellectual property, medicines and technologies;
- build, maintain and operate a commercial-scale cGMP manufacturing facility.

We expect that our cash, cash equivalents and marketable securities at March 31, 2021, will enable us to fund our current and planned operating expenses and capital expenditures for at least the next 12 months. We have based these estimates on assumptions that may prove to be imprecise, and we may exhaust our available capital resources sooner that we currently expect. Because of the numerous risks and uncertainties associated with the development our programs, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates.

Our future funding requirements will depend on many factors including:

- the cost of continuing to build our base editing platform;
- the costs of acquiring licenses for the delivery modalities that will be used with our product candidates;
- the scope, progress, results, and costs of discovery, preclinical development, laboratory testing, manufacturing and clinical trials for the product candidates we may develop;
- the costs of preparing, filing, and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights, and defending intellectual property-related claims;
- the costs, timing, and outcome of regulatory review of the product candidates we may develop;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing, distribution, coverage and reimbursement for any product candidates for which we receive regulatory approval;
- the success of our license agreements and our collaborations;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under any additional collaboration agreements we obtain;
- the payment of success liabilities, should we choose to pay in cash;
- the extent to which we acquire or in-license products, intellectual property, and technologies; and
- the costs of obtaining, building, operating and expanding our manufacturing capacity.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and licensing arrangements. We do not have any committed external source of funds. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends.

If we raise funds through additional collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, or product candidates, or we may have to 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. We can give no assurance that we will be able to secure such additional sources of funds to support our operations, or, if such funds are available to us, that such additional funding will be sufficient to meet our needs.

#### **Contractual obligations**

We enter into contracts in the normal course of business with contract research organizations and other vendors to assist in the performance of our research and development activities and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

During the three months ended March 31, 2021, we entered into the second phase of our April 2019 lease for office and laboratory space to be built. The minimum of undiscounted lease payments due under the second phase of this lease is \$42.7 million.

In May 2021, the first success payment measurements occurred and success payments to Harvard and Broad Institute were calculated to be \$15.0 million and \$15.0 million, respectively. We elected to make each payment in shares of our common stock, and thereby expect to issue shares of our common stock to settle these liabilities to Harvard and Broad Institute in June 2021.

#### **Off-balance sheet arrangements**

We did not have during the periods presented and we do not currently have any off-balance sheet arrangements, as defined under the applicable regulations of the SEC.

#### Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related to changes in interest rates. As of March 31, 2021, we had cash, cash equivalents, and marketable securities of \$503.5 million, which consisted of cash, money market funds, commercial paper and corporate notes, U.S. Treasury securities and government securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term marketable securities. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, we believe an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio. We have the ability to hold our investments until maturity, and therefore, we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investment portfolio.

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we do contract with vendors that are located outside of the United States and may be subject to fluctuations in foreign currency rates. We may enter into additional contracts with vendors located outside of the United States in the future, which may increase our foreign currency exchange risk.

#### Item 4. Controls and Procedures.

#### Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. 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.

Based on the evaluation of our disclosure controls and procedures as of March 31, 2021, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date are effective at the reasonable assurance level.

#### Changes in Internal Control over Financial Reporting

We continuously seek to improve the efficiency and effectiveness of our internal controls. This results in refinements to processes throughout our company. There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended March 31, 2021 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting. We have not experienced any material impact in our internal controls over financial reporting despite our employees working remotely due to the COVID-19 pandemic. We are continually monitoring and assessing the COVID-19 pandemic to determine any potential impacts on our internal controls over financial reporting including changes to their design and operating effectiveness.



#### PART II. OTHER INFORMATION

#### **Item 1. Legal Proceedings**

We are not currently subject to any material legal proceedings.

#### Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. For a detailed discussion of the risks that affect our business, please refer to the section titled "Item 1A. Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2020. The COVID-19 pandemic may also have the effect of heightening many of the other risks described in the section titled "Item 1A. Risk Factors" in each of our 2020 Form 10-K and quarterly reports, such as risks related to our need to raise additional funding, fluctuation of our quarterly financial results, and our ability to obtain regulatory approvals for our product candidates.

The risk factors set forth below represents new risk factors or those containing changes, including material changes, to the similarly titled risk factor included in "Item 1.A Risk Factors" of our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the SEC on March 15, 2021.

We face significant competition in an environment of rapid technological change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize any product candidates we may develop.

The development and commercialization of new drug products is highly competitive. Moreover, the base editing field is characterized by rapidly changing technologies, significant competition, and a strong emphasis on intellectual property. We will face competition with respect to any product candidates that we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we have research programs. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches.

There are several other companies utilizing CRISPR/Cas9 nuclease technology, including Caribou Biosciences, Editas Medicine, CRISPR Therapeutics, and Intellia Therapeutics. Several additional companies utilize other nuclease-based genome editing technologies, including Zinc Fingers, Arcuses, and TAL Nucleases, which includes Sangamo Biosciences, Precision BioSciences, bluebird bio, Allogene Therapeutics, and Cellectis. Additionally, newer genome editing modalities are emerging, including Prime Medicine, Tessera Therapeutics, Shape Therapeutics, Scribe Therapeutics, PerkinElmer (formerly Horizon Discovery), and Intellia Therapeutics. PerkinElmer and Intellia Therapeutics are developing base editing technology and Tessera Therapeutics is utilizing mobile genetic elements for gene editing. In addition, we face competition from companies utilizing gene therapy, oligonucleotides, and CAR-T therapeutic approaches.

Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates we may develop. This may include other types of therapies, such as small molecule, antibody, and/or protein therapies.

Many of our current or potential competitors, either alone or with their collaboration partners, may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology, and gene therapy industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize product candidates that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any product candidates that we may develop or that would render any product candidates that we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidates that we may develop and commercialize.

Our owned patent applications and in-licensed patents and patent applications and other intellectual property may be subject to priority disputes or to inventorship disputes and similar proceedings. If we or our licensors are unsuccessful in any of these proceedings, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or to cease the development, manufacture, and commercialization of one or more of the product candidates we may develop, which could have a material adverse impact on our business.

Although we have an option to exclusively license certain patents and patent applications directed to Cas9 and Cas12a from Editas, who in turn has licensed such patents from various academic institutions including the Broad Institute, we do not currently have a license to such patents and patent applications. Certain of the U.S. patents and one U.S. patent application to which we hold an option are co-owned by the Broad Institute and MIT, and in some cases co-owned by the Broad Institute, MIT, and Harvard, which we refer to together as the Boston Licensing Parties, and were involved in U.S. interference No. 106,048 with one U.S. patent application co-owned by the University of California, the University of Vienna, and Emmanuelle Charpentier, which we refer to together as the University of California. On September 10, 2018, the Court of Appeals for the Federal Circuit, or the CAFC, affirmed the Patent Trial and Appeal Board of the USPTO's, or PTAB's, holding that there was no interference-in-fact. An interference is a proceeding within the USPTO to determine priority of invention of the subject matter of patent claims filed by different parties.

On June 24, 2019, the PTAB declared an interference (U.S. Interference No. 106,115) between 10 U.S. patent applications ((U.S. Serial Nos. 15/947,680; 15/947,700; 15/947,718; 15/981,807; 15/981,808; 15/981,809; 16/136,159; 16/136,165; 16/136,168; and 16/136,175) that are co-owned by the University of California, and 13 U.S. patents and one U.S. patent application (U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; 8,999,641; and 9,840,713, and U.S. Serial No. 14/704,551)) that are co-owned by the Boston Licensing Parties, which we have an option to under the Editas License Agreement. In the declared interference, the University of California has been designated as the junior party and the Boston Licensing Parties have been designated as the senior party.

As a result of the declaration of interference, an adversarial proceeding in the USPTO before the PTAB has been initiated, which is declared to ultimately determine priority, specifically and which party was first to invent the claimed subject matter. Following oral arguments on the parties' motions in May 2020, the PTAB issued a decision in September 2020, which included, in part, denying the Boston Licensing Parties motion that the University of California should be estopped in the current proceeding by the PTAB's decision in the prior interference proceeding between the parties (No. 106,048), finding that the Boston Licensing Parties remain the senior party in the proceeding, and holding that the interference will proceed to the second, priority phase. An interference is typically divided into two phases. The first phase is referred to as the motions or preliminary motions phase while the second is referred to as the priority phase. In the first phase, each party may raise issues including but not limited to those relating to the patentability of a party's claims based on prior art, written description, and enablement. A party also may seek an earlier priority benefit or may challenge whether the declaration of interference was proper in the first place. Priority, or a determination of who first invented the commonly claimed invention, is determined in the second phase of an interference. Although we cannot predict with any certainty how long the priority phase will actually take, it may take approximately a year or longer before a decision is made by the PTAB. The 10 University of California patent applications and the 13 U.S. patents and one U.S. patent application co-owned by the Boston Licensing Parties involved in U.S. Interference No. 106,115 generally relate to CRISPR/Cas9 systems or eukaryotic cells comprising CRISPR/Cas9 systems having fused or covalently linked RNA and the use thereof in eukaryotic cells. There can be no assurance that the U.S. interference will be resolved in favor of the Boston Licensing Parties. If the U.S. interference resolves in favor of University of California, or if the Boston Licensing Parties' patents and patent application are narrowed, invalidated, or held unenforceable, we may lose the ability to license the optioned patents and patent application and our ability to commercialize our product candidates may be adversely affected if we cannot obtain a license to relevant third party patents that cover our product candidates. We may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be nonexclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our base editing platform technology or product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

We or our licensors may be subject to similar interferences in the future with the same risks as described above. For example, on December 14, 2020, the PTAB declared an interference (U.S. Interference No. 106,126) between 14 U.S. patents and two U.S. patent applications (U.S. Patent Nos. 8,697,359; 8,771,945; 8,795,965; 8,865,406; 8,871,445; 8,889,356; 8,889,418; 8,895,308; 8,906,616; 8,932,814; 8,945,839; 8,993,233; 8,999,641; and 9,840,713, and U.S. Serial Nos. 14/704,551 and 15/330,876) that are co-owned by the Boston Licensing Parties, which we have an option to under the Editas License Agreement, and one U.S. patent application (U.S. Serial Nos. 14/685,510) that is owned by Toolgen, Inc, or Toolgen. In the declared interference, the Boston Licensing Parties have been designated as the junior party and Toolgen has been designated as the senior party. In March 2021, the PTAB issued an order on preliminary motions, granting, in part, and denying, in part, certain motions proposed by the Boston Licensing Parties and Toolgen. Although we cannot predict with any certainty how long the preliminary motions phase will actually take, it may take approximately a year or longer before a decision on the motions is made by the PTAB. The 14 U.S. patents and two U.S. patent applications co-owned by the Boston Licensing Parties involved in U.S. Interference No. 106,126 generally relate to CRISPR/Cas9 systems or eukaryotic cells comprising CRISPR/Cas9 systems having fused or covalently linked RNA and the use thereof in eukaryotic cells.



We or our licensors may also be subject to claims that former employees, collaborators, or other third parties have an interest in our owned patent applications or in-licensed patents or patent applications or other intellectual property as an inventor or co-inventor. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patent applications, such co-owners may be able to license their rights to other third parties, including our competitors. In addition, we may need the cooperation of any such co-owners to enforce any patents that issue from such patent applications against third parties, and such cooperation may not be provided to us.

If we or our licensors are unsuccessful in any interference proceedings or other priority, validity (including any patent oppositions), or inventorship disputes to which we or they are subject, we may lose valuable intellectual property rights through the loss of one or more of our owned, licensed, or optioned patents, or such patent claims may be narrowed, invalidated, or held unenforceable, or through loss of exclusive ownership of or the exclusive right to use our owned or in-licensed patents. In the event of loss of patent rights as a result of any of these disputes, we may be required to obtain and maintain licenses from third parties, including parties involved in any such interference proceedings or other priority or inventorship disputes. Such licenses may not be available on commercially reasonable terms or at all, or may be non-exclusive. If we are unable to obtain and maintain such licenses, we may need to cease the development, manufacture, and commercialization of one or more of the product candidates we may develop. The loss of exclusivity or the narrowing of our patent claims could limit our ability to stop others from using or commercializing similar or identical technology and product candidates. Even if we or our licensors are successful in an interference proceeding or other similar priority or inventorship disputes, it could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could result in a material adverse effect on our business, financial condition, results of operations, or prospects.

# The intellectual property landscape around genome editing technology, including base editing, is highly dynamic, and third parties may initiate legal proceedings alleging that we are infringing, misappropriating, or otherwise violating their intellectual property rights, the outcome of which would be uncertain and may prevent, delay or otherwise interfere with our product discovery and development efforts.

The field of genome editing, especially in the area of base editing technology, is still in its infancy, and no such product candidates have reached the market. Due to the intense research and development that is taking place by several companies, including us and our competitors, in this field, the intellectual property landscape is evolving and in flux, and it may remain uncertain for the coming years. There may be significant intellectual property related litigation and proceedings relating to our owned and in-licensed, and other third party, intellectual property and proprietary rights in the future.

Our commercial success depends upon our ability and the ability of our collaborators and licensors to develop, manufacture, market, and sell any product candidates that we may develop and use our proprietary technologies without infringing, misappropriating, or otherwise violating the intellectual property and proprietary rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive litigation regarding patents and other intellectual property rights as well as administrative proceedings for challenging patents, including interference, derivation, *inter partes* review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be subject to and may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our base editing platform technology and any product candidates we may develop, including interference proceedings, post-grant review, *inter partes* review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions such as oppositions before the EPO. Numerous U.S. and foreign issued patents and pending patent applications that are owned by third parties exist in the fields in which we are developing our product candidates and they may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit.

As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our base editing platform technology and product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of therapies, products or their methods of use or manufacture. We are aware of certain third-party patent applications that, if issued, may be construed to cover our base editing technology and product candidates. There may also be third-party patents of which we are currently unaware with claims to technologies, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents.

Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. Our product candidates make use of CRISPR-based technology, which is a field that is highly active for patent filings. In November 2018, it was reported that 211 patent families and 1835 patent family members worldwide referenced CRISPR or Cas in the title, abstracts or claims. The extensive patent filings related to CRISPR and Cas make it difficult for us to assess the full extent of relevant patents and pending applications that may cover our base editing platform technology and product candidates and their use or manufacture. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our base editing platform technology and product candidates. For example, we are aware of a patent portfolio that is co-owned by the University of California, University of Vienna and Emmanuelle Charpentier, or the University of California Portfolio, which contains multiple patents and pending applications directed to gene editing. The University of California portfolio includes, for example, U.S. Patent Nos. 10,266,850; 10,227,611; 10,000,772; 10,113,167; 10,301,651; 10,308,961; 10,337,029; 10,351,878; 10,407,697; 10,358,659; 10,358,658; 10,385,360; 10,400,253; 10,421,980; 10,428,352; 10,415,061; 10,443,076; 10,487,341; 10,513,712; 10,519,467; 10,526,619; 10,533,190; 10,550,407; 10,563,227; 10,570,419; 10,577,631; 10,597,680; 10,612,045; 10,626,419; 10,640,791; 10,669,560; 10,676,759; 10,752,920; 10,774,344; 10,793,878, 10,900,054; 10,982,230; 10,982,231, which are expected to expire around March 2033, excluding any additional term for patent term adjustment, or PTA, or patent term extension, or PTE, and any disclaimed term for terminal disclaimers. The University of California portfolio also includes numerous additional pending patent applications. If these patent applications issue as patents, they are expected to expire around March 2033, excluding any PTA, PTE, and any disclaimed term for terminal disclaimers. As discussed above, certain applications in the University of California Portfolio are currently subject to U.S. Interference No. 106,115 with certain U.S. patents and one U.S. patent application that are co-owned by the Boston Licensing Parties to which we have an option under the Editas License Agreement. Although we have an option to exclusively license certain patents and patent applications directed to Cas9 and Cas12a from Editas, who in turn has licensed such patents from various academic institutions including Broad Institute, we do not currently have a license to such patents and patent applications. Certain members of the University of California Portfolio are being opposed in Europe by multiple parties. For example, the EPO Opposition Division has initiated opposition proceedings against European Patent Nos. EP3,241,902 B1, EP2,800,811 B1, and EP3,401,400 B1, which are estimated to expire in March 2033 (excluding any patent term adjustments or extensions). The opposition procedure before the EPO allows one or more third parties to challenge the validity of a granted European patent within nine months after grant date of the European patent. Opposition proceedings may involve issues including, but not limited to, priority, patentability of the claims involved, and procedural formalities related to the filing of the patent application. As a result of the opposition proceedings, the Opposition Division can revoke a patent, maintain the patent as granted, or maintain the patent in an amended form. Most of the claims of European Patent No. EP2,800,811 B1 were maintained without amendment by the Opposition Division, but this decision is being appealed. European Patent No. EP3,241,902 B1 was revoked by the Opposition Division, but this decision is being appealed. It is uncertain when or in what manner the Opposition Division will act on the opposition proceedings of European Patent No. EP3,401,400 B1 and how oppositions filed against it will be resolved. If these patents are maintained by the Opposition Division with claims similar to those that are currently opposed, our ability to commercialize our product candidates may be adversely affected if we do not obtain a license to these patents. We may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be nonexclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our base editing platform technology or product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business.

Numerous other patents and patent applications have been filed by other third parties directed to gene editing, guide nucleic acids, PAM sequence variants, split inteins, Cas12b or gene editing in the context of immune therapy or chimeric antigen receptors.

Because of the large number of patents issued and patent applications filed in our field, third parties may allege they have patent rights encompassing our product candidates, technologies or methods. Third parties may assert that we are employing their proprietary technology without authorization and may file patent infringement claims or lawsuit against us, and if we are found to infringe such third-party patents, we may be required to pay damages, cease commercialization of the infringing technology, or obtain a license from such third parties, which may not be available on commercially reasonable terms or at all.

Our ability to commercialize our product candidates in the United States and abroad may be adversely affected if we cannot obtain a license on commercially reasonable terms to relevant third-party patents that cover our product candidates or base editing platform technology. Even if we believe third-party intellectual property claims are without merit, there is no assurance that a court would find in our favor on questions of infringement, validity, enforceability, or priority. A court of competent jurisdiction could hold that these third-party patents are valid, enforceable, and infringed, which could materially and adversely affect our ability to commercialize any product candidates we may develop and any other product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's intellectual property rights, and we are unsuccessful in demonstrating that such patents are invalid or unenforceable, we could be required to obtain a license from such third party to continue developing, manufacturing, and marketing any product candidates we may develop and our technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, we may be unable to commercialize our base editing platform technology or product candidates or such commercialization efforts may be significantly delayed, which could in turn significantly harm our business. We also could be forced, including by court order, to cease developing, manufacturing, and commercializing the infringing technology or product candidates. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar material adverse effect on our business, financial condition, results of operations, and prospects.

Defense of third-party claims of infringement of misappropriation, or violation of intellectual property rights involves substantial litigation expense and would be a substantial diversion of management and employee time and resources from our business. Some third parties may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, financial condition, results of operations and prospects. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Any of the foregoing events could have a material adverse effect on our business and prospects.

#### Item 2. Unregistered Sales of Equity Securities and Uses of Proceeds

#### Use of proceeds from registered securities

In February 2020, we closed our IPO in which we issued and sold 12,176,471 shares of our common stock, including 1,588,235 shares of common stock sold pursuant to the underwriters' full exercise of their option to purchase additional shares, at a public offering price of \$17.00 per share, for aggregate gross proceeds of \$207.0 million. All of the shares issued and sold in the IPO were registered under the Securities Act pursuant to a Registration Statement on Form S-1 (File No. 333-233985), which was declared effective by the SEC on February 5, 2020, and a Registration Statement on Form S-1 MEF (File No. 333-236284) filed pursuant to Rule 462(b) of the Securities Act. The offering commenced on February 5, 2020 and did not terminate until the sale of all the shares offered.

Our use of the net offering proceeds through the date of the filing of this Quarterly Report on Form 10-Q, is consistent with the use of proceeds described in our final prospectus filed with the SEC pursuant to Rule 424(b)(4) on February 7, 2020, and there has been no material change in our planned use of the balance of the net proceeds from our IPO described in such prospectus.

#### Item 6. Exhibits.

Exhibit <u>Number</u>	Description of Exhibit	If Incorporated by Reference				
		Form	File Number	Date of Filing	Exhibit Number	Filed Herewith
3.1	Fourth Amended Certificate of Incorporation of Beam Therapeutics Inc.	8-K	001-39208	02/11/2020	3.1	
3.2	Amended and Restated By-laws of Beam Therapeutics Inc.	8-K	001-39208	02/11/2020	3.2	
4.1	Form of Purchase Agreement, dated as of January 16, 2021, among Beam Therapeutics Inc. and each purchaser party thereto	8-K	001-39208	01/19/2021	10.1	
31.1	Certification of Principal Executive Officer Pursuant to Rules <u>13a-14(a) and 15d-14(a) under the Securities Exchange Act</u> of 1934, as Adopted Pursuant to Section 302 of the Sarbanes- Oxley Act of 2002.					Х
31.2	Certification of Principal Financial Officer Pursuant to Rules <u>13a-14(a) and 15d-14(a) under the Securities Exchange Act</u> of 1934, as Adopted Pursuant to Section 302 of the Sarbanes- Oxley Act of 2002.					Х
32.1	<u>Certification of Principal Executive Officer Pursuant to 18</u> <u>U.S.C. Section 1350, as Adopted Pursuant to Section 906 of</u> <u>the Sarbanes-Oxley Act of 2002.</u>					Х
32.2	<u>Certification of Principal Financial Officer Pursuant to 18</u> <u>U.S.C. Section 1350, as Adopted Pursuant to Section 906 of</u> <u>the Sarbanes-Oxley Act of 2002.</u>					Х
101.INS	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					Х
101.SCH	Inline XBRL Taxonomy Extension Schema Document					Х
101.CAL	Inline XBRL Taxonomy Calculation Linkbase Document					Х
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document					Х
101.LAB	Inline XBRL Taxonomy Label Linkbase Document					Х
101.PRE	Inline XBRL Taxonomy Presentation Linkbase Document					Х
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)					Х

#### 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.

BEAM THERAPEUTICS INC.

By:

Date: May 11, 2021

**Chief Executive Officer** (Principal executive officer)

By:

/s/ Terry-Ann Burrell Terry-Ann Burrell Chief Financial Officer and Treasurer (Principal financial and accounting officer)

/s/ John Evans
John Evans

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Date: May 11, 2021

#### CERTIFICATION PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, John Evans, certify that:

- 1. I have reviewed this Quarterly Report on Form 10-Q of Beam Therapeutics Inc.;
- 2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
- 3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
- 4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
- 5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 11, 2021

By: /s/ John Evans

John Evans Chief Executive Officer (Principal executive officer)

#### CERTIFICATION PURSUANT TO RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Terry-Ann Burrell, certify that:

- 1. I have reviewed this Quarterly Report on Form 10-Q of Beam Therapeutics Inc.;
- 2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
- 3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
- 4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
- 5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 11, 2021

By: /s/ Terry-Ann Burrell

Terry-Ann Burrell Chief Financial Officer (Principal financial and accounting officer)

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

In connection with this Quarterly Report of Beam Therapeutics Inc. (the "Company") on Form 10-Q for the period ending March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 11, 2021

By: /s/ John Evans

John Evans Chief Executive Officer (Principal executive officer)

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

In connection with this Quarterly Report of Beam Therapeutics Inc. (the "Company") on Form 10-Q for the period ending March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

- (1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 11, 2021

By: /s/ Terry-Ann Burrell

Terry-Ann Burrell Chief Financial Officer (Principal financial and accounting officer)