
**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
WASHINGTON, D.C. 20549

FORM 10-Q

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

For the quarterly period ended September 30, 2020

OR

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

For the transition period from _____ to _____

Commission File Number: 001-39319

GENERATION BIO CO.

(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

81-4301284
(I.R.S. Employer
Identification Number)

301 Binney Street
Cambridge, Massachusetts
(Address of principal executive offices)

02142
(Zip Code)

(617) 655-7500

(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 Par Value	GBIO	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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post 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

Accelerated filer

Non-accelerated filer

Smaller reporting company

Emerging growth company

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

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

As of October 31, 2020 there were 46,516,475 shares of Common Stock, \$0.0001 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or this Quarterly Report, contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act and Section 21E of the Securities Exchange Act of 1934, as amended, that involve substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this Quarterly Report, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would,” or the negative of these words or other similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Quarterly Report include, among other things, statements about:

- the initiation, timing, progress and results of our research and development programs and preclinical studies and clinical trials;
- the impact of the COVID-19 pandemic and our response to the pandemic;
- our estimates regarding expenses, future revenue, capital requirements, need for additional financing and the period over which we believe that our existing cash, cash equivalents and marketable securities will be sufficient to fund our operating expenses and capital expenditure requirements;
- our plans to develop and, if approved, subsequently commercialize any product candidates we may develop;
- the timing of and our ability to submit applications for, obtain and maintain regulatory approvals for any product candidates we may develop;
- the potential advantages of our non-viral gene therapy platform;
- our estimates regarding the potential addressable patient populations for our programs;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our expectations regarding our ability to obtain and maintain intellectual property protection;
- our intellectual property position;
- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives;
- the impact of government laws and regulations;
- our competitive position and expectations regarding developments and projections relating to our competitors and any competing therapies that are or become available;
- developments and expectations regarding developments and projections relating to our competitors and our industry;
- our ability to maintain and establish collaborations or obtain additional funding; and
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startup Act of 2012.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report, particularly in the “Risk Factors” section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Moreover, we operate in a competitive and rapidly changing environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures, or investments we may make or enter into.

You should read this Quarterly Report and the documents that we file with the Securities and Exchange Commission with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report are made as of the date of this Quarterly Report, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

Generation Bio Co.

INDEX

PART I – FINANCIAL INFORMATION

Page(s)

Item 1. Financial Statements (unaudited)	4
Condensed Consolidated Balance Sheets	4
Condensed Consolidated Statements of Operations and Comprehensive Loss	5
Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)	6
Condensed Consolidated Statements of Cash Flows	8
Notes to Condensed Consolidated Financial Statements	9
Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations	17
Item 3. Quantitative and Qualitative Disclosures About Market Risk	25
Item 4. Controls and Procedures	26

PART II – OTHER INFORMATION

Item 1. Legal Proceedings	26
Item 1A. Risk Factors	26
Item 2. Unregistered Sales of Equity Securities and Use of Proceeds	76
Item 6. Exhibits	76
Signatures	77

PART I—FINANCIAL INFORMATION
Item 1. Financial Statements (unaudited)

Generation Bio Co.
Condensed Consolidated Balance Sheets
(In thousands, except share and per share amounts)
(Unaudited)

	<u>September 30, 2020</u>	<u>December 31, 2019</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 58,605	\$ 15,076
Marketable securities	221,761	—
Tenant receivable	—	448
Prepaid expenses and other current assets	5,447	2,577
Restricted cash	—	55
Total current assets	285,813	18,156
Property and equipment, net	22,888	21,845
Restricted cash, noncurrent	2,052	2,052
Deferred offering costs	—	87
Other long-term assets	169	—
Total assets	<u>\$ 310,922</u>	<u>\$ 42,140</u>
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	\$ 418	\$ 2,251
Accrued expenses and other current liabilities	8,745	6,907
Total current liabilities	9,163	9,158
Deferred rent, net of current portion	15,220	15,981
Total liabilities	24,383	25,139
Commitments and contingencies (Note 9)		
Convertible preferred stock (Series A, B and C), \$0.0001 par value; no shares and 26,425,664 shares authorized at September 30, 2020 and December 31, 2019, respectively; no shares and 26,425,664 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	—	115,593
Stockholders' equity (deficit):		
Preferred stock, \$0.0001 par value; 5,000,000 and no shares authorized at September 30, 2020 and December 31, 2019, respectively; no shares issued or outstanding	—	—
Common stock, \$0.0001 par value; 150,000,000 and 46,750,000 shares authorized at September 30, 2020 and December 31, 2019, respectively; 46,516,475 and 6,976,077 shares issued at September 30, 2020 and December 31, 2019, respectively; 45,559,757 and 5,270,889 shares outstanding at September 30, 2020 and December 31, 2019, respectively	5	1
Additional paid-in capital	451,257	9,859
Accumulated other comprehensive income	26	—
Accumulated deficit	(164,749)	(108,452)
Total stockholders' equity (deficit)	286,539	(98,592)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 310,922</u>	<u>\$ 42,140</u>

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

Generation Bio Co.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share amounts)
(Unaudited)

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
Operating expenses:				
Research and development	\$ 15,308	\$ 13,447	\$ 42,158	\$ 37,403
General and administrative	5,661	2,886	14,611	8,741
Total operating expenses	<u>20,969</u>	<u>16,333</u>	<u>56,769</u>	<u>46,144</u>
Loss from operations	(20,969)	(16,333)	(56,769)	(46,144)
Other income (expense):				
Interest income and other income (expense), net	120	205	472	890
Net loss and net loss attributable to common stockholders	<u>\$ (20,849)</u>	<u>\$ (16,128)</u>	<u>\$ (56,297)</u>	<u>\$ (45,254)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.46)</u>	<u>\$ (3.31)</u>	<u>\$ (2.68)</u>	<u>\$ (9.90)</u>
Weighted average common shares outstanding, basic and diluted	<u>45,468,838</u>	<u>4,866,830</u>	<u>21,011,439</u>	<u>4,571,641</u>
Comprehensive loss:				
Net loss	\$ (20,849)	\$ (16,128)	\$ (56,297)	\$ (45,254)
Other comprehensive income (loss):				
Unrealized gains (losses) on marketable securities	30	(9)	26	11
Comprehensive loss	<u>\$ (20,819)</u>	<u>\$ (16,137)</u>	<u>\$ (56,271)</u>	<u>\$ (45,243)</u>

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

Generation Bio Co.
Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)
(In thousands, except share amounts)
(Unaudited)

	Series A, B and C Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Three Months Ended September 30, 2020								
Balances at June 30, 2020	—	\$ —	45,028,024	\$ 5	\$ 448,052	\$ (4)	\$ (143,900)	\$ 304,153
Issuance of common stock upon exercise of stock options	—	—	279,379	—	1,044	—	—	1,044
Vesting of restricted common stock	—	—	252,354	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	2,161	—	—	2,161
Unrealized gains on marketable securities	—	—	—	—	—	30	—	30
Net loss	—	—	—	—	—	—	(20,849)	(20,849)
Balances at September 30, 2020	—	\$ —	45,559,757	\$ 5	\$ 451,257	\$ 26	\$ (164,749)	\$ 286,539
Three Months Ended September 30, 2019								
Balances at June 30, 2019	26,425,664	115,593	4,615,383	\$ 1	\$ 7,603	\$ 11	\$ (76,261)	\$ (68,646)
Issuance of common stock for license	—	—	125,677	—	—	—	—	—
Vesting of restricted common stock	—	—	248,386	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	1,080	—	—	1,080
Unrealized losses on marketable securities	—	—	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	—	—	(16,128)	(16,128)
Balances at September 30, 2019	26,425,664	\$ 115,593	4,989,446	\$ 1	\$ 8,683	\$ 2	\$ (92,389)	\$ (83,703)

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

Generation Bio Co.
Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)
(In thousands, except share amounts)
(Unaudited)

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Nine Months Ended September 30, 2020								
Balances at December 31, 2019	26,425,664	\$ 115,593	5,270,889	\$ 1	\$ 9,859	\$ —	\$ (108,452)	\$ (98,592)
Issuance of Series C convertible preferred stock, net of issuance costs of \$2,640	19,936,296	108,832	—	—	—	—	—	—
Conversion of convertible preferred stock into common stock upon initial public offering	(46,361,960)	(224,425)	27,094,085	3	224,422	—	—	224,425
Issuance of common stock upon initial public offering, net of issuance costs of \$3,185	—	—	12,105,263	1	210,714	—	—	210,715
Issuance of common stock upon exercise of stock options	—	—	319,642	—	1,229	—	—	1,229
Vesting of restricted common stock	—	—	769,878	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	5,033	—	—	5,033
Unrealized gains on marketable securities	—	—	—	—	—	26	—	26
Net loss	—	—	—	—	—	—	(56,297)	(56,297)
Balances at September 30, 2020	<u>—</u>	<u>\$ —</u>	<u>45,559,757</u>	<u>\$ 5</u>	<u>\$ 451,257</u>	<u>\$ 26</u>	<u>\$ (164,749)</u>	<u>\$ 286,539</u>
Nine Months Ended September 30, 2019								
Balances at December 31, 2018	26,425,664	\$ 115,593	4,054,475	\$ 1	\$ 5,552	\$ (9)	\$ (47,135)	\$ (41,591)
Issuance of common stock for license	—	—	125,677	—	—	—	—	—
Issuance of common stock upon exercise of stock options	—	—	14,861	—	9	—	—	9
Vesting of restricted common stock	—	—	794,433	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	3,122	—	—	3,122
Unrealized gains on marketable securities	—	—	—	—	—	11	—	11
Net loss	—	—	—	—	—	—	(45,254)	(45,254)
Balances at September 30, 2019	<u>26,425,664</u>	<u>\$ 115,593</u>	<u>4,989,446</u>	<u>\$ 1</u>	<u>\$ 8,683</u>	<u>\$ 2</u>	<u>\$ (92,389)</u>	<u>\$ (83,703)</u>

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

Generation Bio Co.
Condensed Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2020	2019
Cash flows from operating activities:		
Net loss	\$ (56,297)	\$ (45,254)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	5,033	3,122
Depreciation and amortization expense	2,441	1,185
Amortization (accretion) of premium (discount) on marketable securities	88	(335)
Changes in operating assets and liabilities:		
Tenant receivable	448	(1,297)
Prepaid expenses and other current assets	(2,870)	(1,078)
Other noncurrent assets	(169)	—
Accounts payable	(1,321)	(132)
Accrued expenses and other current liabilities	1,631	2,284
Deferred rent	(761)	12,966
Net cash used in operating activities	<u>(51,777)</u>	<u>(28,539)</u>
Cash flows from investing activities:		
Purchases of property and equipment	(3,724)	(18,828)
Purchases of marketable securities	(221,823)	(20,789)
Sales and maturities of marketable securities	—	82,680
Net cash provided by (used in) investing activities	<u>(225,547)</u>	<u>43,063</u>
Cash flows from financing activities:		
Proceeds from issuance of convertible preferred stock, net of issuance costs incurred and paid in current period	108,854	—
Proceeds from initial public offering of common stock, net of underwriting discounts and commissions	213,900	—
Proceeds from exercise of stock options	1,229	9
Payment of initial public offering costs	(3,185)	—
Net cash provided by financing activities	<u>320,798</u>	<u>9</u>
Net increase in cash, cash equivalents and restricted cash	43,474	14,533
Cash, cash equivalents and restricted cash at beginning of period	<u>17,183</u>	<u>9,466</u>
Cash, cash equivalents and restricted cash at end of period	<u>\$ 60,657</u>	<u>\$ 23,999</u>
Supplemental disclosure of noncash investing and financing information:		
Conversion of convertible preferred stock to common stock	\$ 224,425	\$ —
Purchases of property and equipment included in accounts payable and accrued expenses	\$ 404	\$ 895

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

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

1. Nature of the Business and Basis of Presentation

The Company was incorporated on October 21, 2016 as Torus Therapeutics, Inc. and subsequently changed its name to Generation Bio Co. (the “Company” or “Generation Bio”). The Company is an innovative genetic medicines company creating a new class of gene therapy utilizing its proprietary non-viral gene therapy platform to provide durable, redosable treatments for millions of patients living with rare and prevalent diseases. The Company’s non-viral gene therapy platform incorporates its high-capacity DNA construct called closed-ended DNA (“ceDNA”), its cell-targeted lipid nanoparticle delivery system (“ctLNP”) and its established, scalable capsid-free manufacturing process. Using its approach, the Company is developing novel gene therapies to provide targeted delivery of genetic payloads that include large and multiple genes to a range of tissues across a broad array of diseases. The Company is also engineering its gene therapies to be redosable, which may enable individualized patient titration to reach the desired therapeutic expression and to maintain efficacy throughout a patient’s life. The Company is headquartered in Cambridge, Massachusetts.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, the ability to establish clinical- and commercial-scale manufacturing processes, the impact of the COVID-19 pandemic and the ability to secure additional capital to fund operations. Programs currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization of a product. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company’s development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

On June 16, 2020, the Company completed its initial public offering (“IPO”) pursuant to which it issued and sold 12,105,263 shares of its common stock, including 1,578,947 shares pursuant to the full exercise of the underwriters’ option to purchase additional shares, at a public offering price of \$19.00 per share, resulting in net proceeds of \$210.7 million, after deducting underwriting discounts and commissions and other offering expenses. Upon the closing of the IPO, all of the Company’s outstanding convertible preferred stock automatically converted into shares of common stock.

The accompanying consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. Since inception, the Company has funded its operations with proceeds from the sales of instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), the sale of convertible preferred stock, and most recently, with proceeds from the sale of common stock in the IPO completed in June 2020. The Company has incurred recurring losses, including net losses of \$56.3 million for the nine months ended September 30, 2020 and \$61.3 million for the year ended December 31, 2019. As of September 30, 2020, the Company had an accumulated deficit of \$164.7 million. The Company expects to continue to generate operating losses in the foreseeable future. As of November 10, 2020, the issuance date of these interim condensed consolidated financial statements, the Company expects that its cash, cash equivalents and marketable securities will be sufficient to fund its operating expenses and capital expenditure requirements for at least 12 months.

The Company will need to obtain additional funding through public or private equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into collaborations or strategic alliances or licensing arrangements. The terms of any financing may adversely affect the holdings or the rights of the Company’s stockholders. Arrangements with collaborators or others may require the Company to relinquish rights to certain of its technologies or programs. If the Company is unable to obtain funding, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, pipeline expansion or commercialization efforts, which could adversely affect its business prospects. Although management will continue to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations when needed or at all.

The accompanying consolidated financial statements reflect the operations of the Company and the Company’s wholly owned subsidiary, Generation Bio Securities Corporation. Intercompany balances and transactions have been eliminated in consolidation. The accompanying consolidated financial statements have been prepared in conformity with generally accepted accounting principles

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

(“GAAP”) in the United States of America. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Update (“ASU”) of the Financial Accounting Standards Board (“FASB”).

2. Summary of Significant Accounting Policies

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 and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting periods. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, the accrual of research and development expenses and stock-based compensation expense. The Company bases its estimates on historical experience, known trends and other market-specific or other relevant factors that it believes to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates, as there are changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results may differ from those estimates or assumptions.

Unaudited interim financial information

The balance sheet at December 31, 2019 was derived from audited financial statements but does not include all disclosures required by GAAP. The accompanying unaudited financial statements as of September 30, 2020 and for the three and nine months ended September 30, 2020 and 2019 have been prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission (“SEC”) for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. These financial statements should be read in conjunction with the Company’s audited financial statements included in the Company’s Registration Statement on Form S-1 (File No. 333-238608) on file with SEC. In the opinion of management, all adjustments, consisting only of normal recurring adjustments necessary for a fair presentation of the Company’s financial position as of September 30, 2020, the results of operations for the three and nine months ended September 30, 2020 and 2019, and cash flows for the nine months ended September 30, 2020 and 2019 have been made. The results of operations for the three and nine months ended September 30, 2020 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2020 or any other period.

Concentrations of credit risk and of significant suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents, and marketable securities. The Company believes it is not exposed to significant credit risk due to the financial strength of the depository institutions in which the cash and cash equivalents are held. The Company maintains its cash equivalents in money market funds that invest in U.S. treasury securities. The Company’s marketable securities as of September 30, 2020 consisted of U.S government treasury securities, U.S. government agency bonds, commercial paper and corporate debt securities. The Company has adopted an investment policy that limits the amounts the Company may invest in the securities of single issuer with the exclusion of the U.S. government. The Company has not experienced any credit losses and does not believe that it is subject to significant credit risk.

The Company is dependent on a small number of third-party suppliers for its drug substance and drug product. In particular, the Company relies, and expects to continue to rely, on third-party suppliers for certain materials and components required for the production of any product candidates it may develop for its programs. These programs could be adversely affected by a significant interruption in the supply process.

Fair value measurements

Certain assets and liabilities of the Company are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and financial liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and marketable securities are carried at fair value, determined according to the fair value hierarchy described above (see Note 3). The carrying values of the Company's accounts payable and accrued expenses approximate their fair values due to the short-term nature of these liabilities.

Recently adopted accounting pronouncements

In August 2018, the FASB issued ASU 2018-13, Fair Value Measurement (Topic 820): Disclosure Requirements for Fair Value Measurement. The new standard added, modified or removed disclosure requirements under Topic 820 for clarity and consistency. ASU 2018-13 is effective for all entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. The Company adopted ASU 2018-03 on January 1, 2020 and the adoption did not have a material impact on its consolidated financial statements.

Recently issued accounting pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies that the Company adopts as of the specified effective date. The Company qualifies as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012 and has elected not to "opt out" of the extended transition related to complying with new or revised accounting standards, which means that when a standard is issued or revised and it has different application dates for public and nonpublic companies, the Company can adopt the new or revised standard at the time nonpublic companies adopt the new or revised standard and can do so until such time that the Company either (i) irrevocably elects to "opt out" of such extended transition period or (ii) no longer qualifies as an emerging growth company.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). The new standard requires that all lessees recognize the assets and liabilities that arise from leases on the balance sheet and disclose qualitative and quantitative information about its leasing arrangements. In July 2018, the FASB issued ASU 2018-11, which provides entities with an additional transition method to adopt Topic 842. Under the new transition method, an entity initially applies the new lease requirements at the adoption date, not the earliest period presented, and recognizes a cumulative effect adjustment to the opening balance of retained earnings in the period of adoption. For public entities, the guidance was effective for annual reporting periods beginning after December 15, 2018 and for interim periods within those fiscal years. For nonpublic entities, the guidance was effective for annual reporting periods beginning after December 15, 2019. Early adoption is permitted for all entities. In November 2019, the FASB issued ASU 2019-10, which deferred the effective date for nonpublic entities to annual reporting periods beginning after December 15, 2020, and interim periods within fiscal years beginning after December 15, 2021. In June 2020, the FASB issued ASU 2020-05, which grants a one-year effective-date delay for nonpublic entities to annual reporting periods beginning after December 15, 2021 and to interim periods within fiscal years beginning after December 15, 2022. Early adoption continues to be allowed. The Company plans to adopt ASU 2016-02 on January 1, 2021 using the modified retrospective approach transition method as of the date of adoption such that prior periods will not be restated. The Company will also elect a package of practical expedients, under which an entity need not reassess whether any expired or existing contracts are or contain leases, the lease classification for any expired or existing leases, or initial direct costs for any existing leases. The adoption of the new standard is expected to result in the recognition of material liabilities and right-of-use assets, however, the Company has not completed its assessment.

In June 2016, the FASB issued ASU 2016-13, Financial Instruments – Credit Losses (Topic 326). The new standard adjusts the accounting for assets held at amortized costs basis, including marketable securities accounted for as available-for-sale. The standard eliminates the probable initial recognition threshold and requires an entity to reflect its current estimate of all expected credit losses. The allowance for credit losses is a valuation account that is deducted from the amortized cost basis of the financial assets to present the net amount expected to be collected. For public entities, the guidance is effective for annual reporting periods beginning after December 15, 2019 and for interim periods within those fiscal years. For nonpublic entities, the guidance was effective for annual reporting periods beginning after December 15, 2020. Early adoption is permitted for all entities. In November 2019, the FASB issued

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

ASU 2019-10, which deferred the effective date for nonpublic entities to annual reporting periods beginning after December 15, 2022, including interim periods within those fiscal years. The Company is currently evaluating when to adopt the guidance and the impact the guidance will have on its consolidated financial statements.

3. Fair Value of Financial Assets

As of September 30, 2020, marketable securities by security type consisted of the following:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
	(in thousands)			
U.S. treasury securities	\$ 132,184	\$ 16	\$ —	\$ 132,200
U.S. government agency bonds	11,995	2	—	11,997
Commercial paper	59,891	—	—	59,891
Corporate debt securities	17,665	8	—	17,673
Totals	\$ 221,735	\$ 26	\$ —	\$ 221,761

As of September 30, 2020, marketable securities consisted of investments that mature within one year. The Company did not have marketable securities at December 31, 2019.

The following tables present the Company's assets that are measured at fair value on a recurring basis and indicate the level within the fair value hierarchy of the valuation techniques the Company utilized to determine such fair value, which is described further within Note 2, Summary of Significant Accounting Policies:

	Fair Value Measurements at September 30, 2020 Using:			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
Cash equivalents:				
Money market funds	\$ 57,394	\$ —	\$ —	\$ 57,394
Marketable securities:				
U.S. treasury securities	—	132,200	—	132,200
U.S. government agency bonds	—	11,997	—	11,997
Commercial paper	—	59,891	—	59,891
Corporate debt securities	—	17,673	—	17,673
Totals	\$ 57,394	\$ 221,761	\$ —	\$ 279,155

	Fair Value Measurements at December 31, 2019 Using:			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
Cash equivalents:				
Money market funds	\$ 15,076	\$ —	\$ —	\$ 15,076
Totals	\$ 15,076	\$ —	\$ —	\$ 15,076

Money market funds were valued based on quoted market prices, which represent a Level 1 measurement within the fair value hierarchy. The Company's marketable securities, which consisted of U.S. treasury securities, U.S. government agency bonds, commercial paper and corporate debt securities were valued using quoted prices in active markets for similar securities, which represent a Level 2 measurement within the fair value hierarchy.

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

4. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	September 30, 2020	December 31, 2019
	(in thousands)	
Accrued employee compensation and benefits	\$ 3,798	\$ 3,255
Accrued external research and development expenses	2,037	1,344
Accrued professional fees	890	619
Deferred rent	1,380	1,370
Other	640	319
	<u>\$ 8,745</u>	<u>\$ 6,907</u>

5. Convertible Preferred Stock

The Company had issued Series A convertible preferred stock (the “Series A”), Series B convertible preferred stock (the “Series B”) and Series C convertible preferred stock (the “Series C”). Collectively the Series A, Series B and Series C are referred to as the Preferred Stock.

On January 9, 2020, the Company issued and sold 19,936,296 shares of Series C at a price of \$5.5914 per share for gross proceeds of \$111.5 million. The Company incurred issuance costs in connection with this transaction of \$2.6 million.

Upon the closing of the IPO in June 2020, the Company’s Preferred Stock automatically converted into 27,094,085 shares of common stock.

6. Equity

As of September 30, 2020, the Company’s amended and restated certificate of incorporation authorizes the Company to issue 150,000,000 shares of common stock, par value \$0.0001 per share, and 5,000,000 shares of preferred stock, par value \$0.0001 per share, all of which preferred stock is undesignated.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company’s stockholders. Common stockholders are not entitled to receive dividends, unless declared by the board of directors.

7. Stock-Based Awards

Stock incentive plans

The Company’s 2017 Stock Incentive Plan (the “2017 Plan”) provided for the Company to grant incentive stock options or nonstatutory stock options, restricted stock, restricted stock units and other equity awards to employees, directors and consultants of the Company. In January 2020, the number of shares of common stock authorized for issuance under the 2017 Plan was increased from 8,407,405 shares to 10,275,717 shares.

In May 2020 the Company’s board of directors adopted, and in June 2020 the Company’s stockholders approved, the 2020 Stock Incentive Plan (the “2020 Plan” and, together with the 2017 Plan the “Plans”), which became effective on June 11, 2020. The 2020 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Upon effectiveness of the 2020 Plan, the number of shares of common stock reserved for issuance under the 2020 Plan was the sum of (1) 2,547,698 shares; plus (2) the number of shares (up to a maximum of 7,173,014 shares) as was equal to the sum of (x) the number of shares of common stock reserved for issuance under the 2017 Plan that remained available for grant under the 2017 Plan on June 11, 2020 and (y) the number of shares of common stock subject to outstanding awards granted under the 2017 Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right; plus (3) an annual increase, to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2021 and continuing until, and including, the fiscal year ending December 31, 2030, equal to the lesser of (i) 4% of the number of shares of common stock outstanding on such date, and (ii) an amount determined by the board of directors. Upon the effectiveness of the 2020 Plan, the Company ceased granting additional awards under the 2017 Plan.

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

The Plans are administered by the board of directors or, at the discretion of the board of directors, by a committee of the board of directors. The exercise prices, vesting and other restrictions on any award under the Plans are determined at the discretion of the board of directors, or its committee if so delegated. Stock options granted under the Plans with service-based vesting conditions generally vest over four years and expire after ten years. The exercise price for stock options granted is not less than the fair value of common stock as of the date of grant. Prior to the Company's IPO, fair value of common stock was determined by the board of directors. Subsequent to the IPO, fair value of common stock will be based on quoted market prices.

As of September 30, 2020, 2,640,244 shares remained available for future issuance under the 2020 Plan. Shares subject to outstanding awards granted under the Plans that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right will be available for future awards under the 2020 Plan.

Grant of stock options

During the nine months ended September 30, 2020, the Company granted service-based options to certain employees, directors and consultants for the purchase of 2,114,361 shares of common stock with a weighted average grant date fair value of \$8.71 per share that vest over approximately four years.

During the nine months ended September 30, 2020, the Company granted performance-based options to certain employees, directors and consultants for the purchase of 717,154 shares of common stock with a weighted average grant date fair value of \$3.26 per share that vest upon the achievement of specified milestones.

Employee stock purchase plan

In May 2020 the Company's board of directors adopted, and in June 2020 the Company's stockholders approved, the 2020 Employee Stock Purchase Plan (the "2020 ESPP"), which became effective June 11, 2020. The 2020 ESPP will be administered by the Company's board of directors or by a committee appointed by the board of directors. The 2020 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of 481,231 shares of common stock. The number of shares of common stock reserved for issuance under the 2020 ESPP will automatically increase on the first day of each fiscal year, beginning with the fiscal year commencing on January 1, 2021 and continuing for each fiscal year until, and including the fiscal year commencing on, January 1, 2030, in an amount equal to the lowest of (1) 1,302,157 shares of common stock, (2) 1% of the number of shares of common stock outstanding on such date, and (3) an amount determined by the board of directors. No offering periods have commenced under the 2020 ESPP and 481,231 shares remain available for issuance.

Stock-based compensation

Stock-based compensation expense was classified in the statements of operations and comprehensive loss as follows:

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	2020	2019	2020	2019
	(in thousands)			
Research and development expenses	\$ 1,204	\$ 688	\$ 2,967	\$ 2,039
General and administrative expenses	957	392	2,066	1,083
	<u>\$ 2,161</u>	<u>\$ 1,080</u>	<u>\$ 5,033</u>	<u>\$ 3,122</u>

As of September 30, 2020, total unrecognized compensation cost related to unvested stock-based awards was \$23.6 million, which is expected to be recognized over a weighted average period of 3.2 years. Additionally, as of September 30, 2020, the Company has unrecognized compensation cost related to unvested stock-based awards with performance-based vesting conditions for which performance has not been deemed probable of \$2.3 million.

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

8. Net Loss per Share

The following potential dilutive securities, presented based on amounts outstanding at each period end, have been excluded from the calculation of diluted net loss per share because including them would have had an anti-dilutive impact:

	September 30,	
	2020	2019
Convertible preferred stock (as converted to common stock)	—	14,961,027
Unvested restricted common stock	956,718	1,970,209
Stock options to purchase common stock	5,581,717	3,171,498
	<u>6,538,435</u>	<u>20,102,734</u>

9. Commitments and Contingencies

The Company leases its office and laboratory space under a noncancelable operating lease that was entered into in August 2018, as amended in June 2020, and expires in 2029. The Company has an option to extend for one additional term of five years at the greater of the then-current base rent, or the then-current fair market value. The lease agreement includes provisions for a free-rent period and annual rent increases, which are accrued or deferred as appropriate such that rent expense for the lease is recognized on a straight-line basis over the lease term. The Company is responsible for real estate taxes, maintenance, and other operating expenses applicable to the leased premises. The lease provides for a tenant improvement allowance, at the cost of the lessor, not to exceed \$14.7 million. Costs incurred by the Company for tenant improvements but not yet reimbursed by the landlord are presented on the accompanying consolidated balance sheets as tenant receivable. The Company posted a customary letter of credit in the amount of approximately \$2.1 million as a security deposit. The letter of credit is subject to increase if the Company were to sublease any portion of the leased premises.

Rent expense for the three months ended September 30, 2020 and 2019 was \$1.8 million and \$2.5 million, respectively. Rent expense for the nine months ended September 30, 2020 and 2019 was \$5.5 million and \$6.0 million, respectively.

Future minimum lease payments as of September 30, 2020 are as follows:

Year Ending December 31,	(in thousands)
2020 (remaining 3 months)	\$ 1,745
2021	7,053
2022	7,251
2023	7,441
2024	7,679
Thereafter	35,451
	<u>\$ 66,620</u>

401(k) Plan

The Company has a defined-contribution plan under Section 401(k) of the Internal Revenue Code of 1986 (the "401(k) Plan"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. In September 2020, the Company adopted a match program for employee contributions to the 401(k) Plan up to a maximum of four percent of the employee's salary for the year ended December 31, 2021.

Indemnification agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, contract research organizations, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and its executive officers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. The Company has not incurred any material costs as a result of such indemnifications and is not currently aware of any indemnification claims.

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

Legal proceedings

The Company, from time to time, may be party to litigation arising in the ordinary course of business. The Company was not subject to any material legal proceedings during the three and nine months ended September 30, 2020.

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 consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q, or Quarterly Report, and our final prospectus for our initial public offering, or IPO, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, or the Securities Act, with the Securities and Exchange Commission, or SEC. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the “Risk Factors” section of this Quarterly Report, our actual results could differ materially from the results described in, or implied by, the forward-looking statements contained in the following discussion and analysis.

Overview

We are an innovative genetic medicines company creating a new class of gene therapy utilizing our proprietary non-viral gene therapy platform to provide durable, redosable treatments for millions of patients living with rare and prevalent diseases. Our non-viral gene therapy platform incorporates our high-capacity DNA construct called closed-ended DNA, or ceDNA; our cell-targeted lipid nanoparticle delivery system, or ctLNP; and our established, scalable capsid-free manufacturing process. Using our approach, we are developing novel gene therapies to provide targeted delivery of genetic payloads that include large and multiple genes to a range of tissues across a broad array of diseases. We are also engineering our gene therapies to be redosable, which may enable individualized patient titration to reach the desired level of therapeutic expression and to maintain efficacy throughout a patient’s life.

We are advancing a broad and expansive portfolio including rare and prevalent diseases of the liver and retina, as well as in skeletal muscle, oncology and the central nervous system by developing discrete ctLNPs, each engineered to reach a different tissue. In parallel, we are developing the constructs and manufacturing capacity to rapidly advance new disease programs in a tissue or therapeutic area once human proof of concept is established.

Our most advanced programs are in the liver and retina. In the liver, we have programs for phenylketonuria, or PKU, and hemophilia A, which are in preclinical development, during which we conduct *in vivo* studies to identify development candidates and assess these candidates in studies to enable an investigational new drug, or IND, application. In the retina, we have programs for Leber’s Congenital Amaurosis 10, or LCA10, and Stargardt disease, which are in lead optimization, during which we seek to identify ceDNA constructs that provide disease relevant expression in an animal model.

We expect to select the final development candidate for each of our PKU and hemophilia A programs and to initiate IND-enabling studies for these programs in 2021, followed by IND applications for these programs in 2022. We anticipate submitting IND applications for additional programs in 2023 and beyond.

Since our inception in October 2016, we have focused substantially all of our resources on building our non-viral gene therapy platform, establishing and protecting our intellectual property portfolio, conducting research and development activities, developing our manufacturing process, organizing and staffing our company, business planning, raising capital and providing general and administrative support for these operations. We do not have any products approved for sale and have not generated any revenue from product sales. To date, we have funded our operations with proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), the sales of convertible preferred stock and, most recently, with proceeds from the sale of common stock in our IPO. On June 16, 2020, we completed our IPO, pursuant to which we issued and sold 12,105,263 shares of our common stock, including 1,578,947 shares sold by us pursuant to the full exercise of the underwriters’ option to purchase additional shares. We received net proceeds of \$210.7 million, after deducting underwriting discounts and commissions and other offering expenses.

Since our inception, we have incurred significant operating losses. Our ability to generate any product revenue or product revenue sufficient to achieve profitability will depend on the successful development and eventual commercialization of one or more product candidates we may develop. For the nine months ended September 30, 2020, we reported net losses of \$56.3 million, and for the year ended December 31, 2019, we reported net losses of \$61.3 million. As of September 30, 2020, we had an accumulated deficit of \$164.7 million. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect that our expenses and capital requirements will increase substantially in connection with our ongoing activities, particularly if and as we:

- continue our current research programs and conduct additional research programs;
- advance any product candidates we identify into preclinical and clinical development;
- expand the capabilities of our proprietary non-viral gene therapy platform;

Table of Contents

- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates for which we may obtain regulatory approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development, future commercialization efforts and operations as a public company.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for any product candidates we may develop. If we obtain regulatory approval for any product candidates we may develop, we expect to incur significant expenses related to developing our commercial capability to support product sales, marketing and distribution. Further, we expect to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding 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/or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements when needed or on terms acceptable to us, we would be required to delay, limit, reduce or terminate our product development or future commercialization of one or more of our product candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

We believe that our existing cash, cash equivalents and marketable securities, will enable us to fund our operating expenses and capital expenditures into 2023. We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong. We could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. See “—Liquidity and Capital Resources.”

COVID-19

In March 2020, COVID-19 was declared a global pandemic by the World Health Organization and to date, the COVID-19 pandemic continues to present a substantial public health and economic challenge around the world. The length of time and full extent to which the COVID-19 pandemic may directly or indirectly impact our business, results of operations and financial condition will depend on future developments that are highly uncertain, subject to change and are difficult to predict. We, our contract development and manufacturing organizations, or CDMOs, and our contract research organizations, or CROs, experienced temporary reductions in the capacity to undertake research-scale production and to execute some preclinical studies. While these operations have since normalized, we, together with our CDMOs and CROs, are closely monitoring the impact of the COVID-19 pandemic on these operations.

We also plan to continue to closely monitor the ongoing impact of the COVID-19 pandemic on our employees and our other business operations. In an effort to provide a safe work environment for our employees, we have, among other things, limited employees in our office and lab facilities to those where on-site presence is needed for their job activities, increased the cadence of sanitization of our office and lab facilities, implemented various social distancing measures in our office and labs including replacing all in-person meetings with virtual interactions, and are providing personal protective equipment for our employees present in our office and lab facilities. We expect to continue to take actions as may be required or recommended by government authorities or as we determine are in the best interests of our employees and other business partners in light of the pandemic.

Components of Our Results of Operations

Operating expenses

Research and development expenses

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

- personnel-related costs, including salaries, benefits and stock-based compensation expense, for employees engaged in research and development functions;
- expenses incurred in connection with our research programs, including under agreements with third parties, such as consultants and contractors and CROs;
- the cost of developing and scaling our manufacturing process and manufacturing drug substance and drug product for use in our research and preclinical studies, including under agreements with third parties, such as consultants and contractors and CDMOs;
- laboratory supplies and research materials;
- facilities, depreciation and amortization and other expenses, which include direct and allocated expenses for rent and maintenance of facilities and insurance; and
- payments made under third-party licensing agreements.

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 related goods are delivered or the services are performed.

Our external research and development expenses consist of costs that include fees and other costs paid to consultants, contractors, CDMOs and CROs in connection with our preclinical and manufacturing activities. We do not allocate our research and development costs to specific programs because costs are deployed across multiple programs and our platform and, as such, are not separately classified. We expect that our research and development expenses will increase substantially as we advance our programs into clinical development and expand our discovery, research and preclinical activities in the near term and in the future. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any product candidates we may develop. The successful development of any of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with product development, including the following:

- the timing and progress of preclinical studies, including IND-enabling studies;
- the number and scope of preclinical and clinical programs we decide to pursue;
- raising additional funds necessary to complete preclinical and clinical development of our product candidates;
- the timing of filing and acceptance of INDs or comparable foreign applications that allow commencement of future clinical trials for our product candidates;
- the successful initiation, enrollment and completion of clinical trials, including under current good clinical practices;
- our ability to achieve positive results from our future clinical programs that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended patient populations of any product candidates we may develop;
- the availability of specialty raw materials for use in production of our product candidates;
- our ability to establish arrangements with third-party manufacturers for clinical supply;
- our ability to establish new licensing or collaboration arrangements;
- the receipt and related terms of regulatory approvals from the U.S. Food and Drug Administration and other applicable regulatory authorities;
- our ability to establish, obtain, maintain, enforce and defend patent, trademark, trade secret protection and other intellectual property rights or regulatory exclusivity for any product candidates we may develop and our technology; and
- our ability to maintain a continued acceptable safety, tolerability and efficacy profile of our product candidates following approval.

A change in the outcome of any of these variables with respect to any product candidates we may develop could significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any product candidates we may develop.

[Table of Contents](#)

General and administrative expenses

General and administrative expenses consist primarily of personnel-related costs, including salaries, benefits and stock-based compensation, for employees engaged in executive, legal, finance and accounting and other administrative functions. General and administrative expenses also include professional fees for legal, patent, consulting, investor and public relations and accounting and audit services as well as direct and allocated facility-related costs.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of our programs and platform. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance, director and officer insurance costs and investor and public relations expenses associated with operating as a public company.

Other income (expense)

Interest income and other income (expense), net

Interest income consists of interest earned on our invested cash balances. We expect our interest income to be lower in 2020 than 2019 as a result of lower interest rates.

Other income (expense) consists of miscellaneous income and expense unrelated to our core operations.

Income taxes

Since our inception, we have not recorded any income tax benefits for the net losses we have incurred or for the research and development tax credits earned in each year, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our net operating loss carryforwards and tax credit carryforwards will not be realized.

As of December 31, 2019, we had federal net operating loss carryforwards of \$91.9 million, which may be available to offset future taxable income, of which \$8.2 million of the total net operating loss carryforwards begin to expire in 2036, while the remaining \$83.7 million do not expire but may be limited in their usage to an annual deduction equal to 80% of annual taxable income. In addition, as of December 31, 2019, we had state net operating loss carryforwards of \$90.6 million, which may be available to offset future taxable income and expire at various dates beginning in 2036. As of December 31, 2019, we also had federal and state research and development tax credit carryforwards of \$4.0 million and \$2.5 million, respectively, which may be available to reduce future tax liabilities and expire at various dates beginning in 2036 and 2032, respectively. Due to our history of cumulative net losses since inception and uncertainties surrounding our ability to generate future taxable income, we have recorded a full valuation allowance against our net deferred tax assets at each balance sheet date.

Results of Operations

Comparison of the three months ended September 30, 2020 and 2019

The following table summarizes our results of operations for the three months ended September 30, 2020 and 2019:

	Three Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
Operating expenses:			
Research and development	\$ 15,308	\$ 13,447	\$ 1,861
General and administrative	5,661	2,886	2,775
Total operating expenses	20,969	16,333	4,636
Loss from operations	(20,969)	(16,333)	(4,636)
Other income (expense):			
Interest income and other income (expense), net	120	205	(85)
Net loss	\$ (20,849)	\$ (16,128)	\$ (4,721)

[Table of Contents](#)

Research and development expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2020 and 2019:

	<u>Three Months Ended September 30,</u>		<u>Change</u>
	<u>2020</u>	<u>2019</u>	
	(in thousands)		
Personnel-related	\$ 4,434	\$ 3,130	\$ 1,304
Stock-based compensation	1,204	688	516
Preclinical and manufacturing	4,955	4,379	576
Facilities	2,355	3,144	(789)
Lab supplies	1,086	1,091	(5)
Consulting and professional services	356	349	7
Other	918	666	252
Total research and development expenses	<u>\$ 15,308</u>	<u>\$ 13,447</u>	<u>\$ 1,861</u>

Research and development expenses were \$15.3 million for the three months ended September 30, 2020 compared to \$13.4 million for the three months ended September 30, 2019. The increases in personnel-related costs and stock-based compensation costs of \$1.3 million and \$0.5 million, respectively, were primarily due to increased headcount in our research and development function. The increase in preclinical and manufacturing expense of \$0.6 million was due primarily to increased preclinical activities. The decrease in facility-related expenses of \$0.8 million was primarily due to decreased rent expense.

General and administrative expenses

The following table summarizes our general and administrative expenses for the three months ended September 30, 2020 and 2019:

	<u>Three Months Ended September 30,</u>		<u>Change</u>
	<u>2020</u>	<u>2019</u>	
	(in thousands)		
Personnel-related	\$ 2,160	\$ 1,143	\$ 1,017
Stock-based compensation	957	392	565
Professional and consultant fees	1,905	872	1,033
Facilities	379	401	(22)
Other	260	78	182
Total general and administrative expenses	<u>\$ 5,661</u>	<u>\$ 2,886</u>	<u>\$ 2,775</u>

General and administrative expenses were \$5.7 million for the three months ended September 30, 2020 compared to \$2.9 million for the three months ended September 30, 2019. The increases in personnel-related costs and stock-based compensation costs of \$1.0 million and \$0.6 million, respectively, were primarily a result of an increase in headcount in our general and administrative function. Professional and consultant fees increased by \$1.0 million primarily due to professional fees relating to accounting, audit and legal services as well as costs associated with ongoing business activities and our preparations to operate as a public company.

Other income and expense, net

Other income and expense, net for the three months ended September 30, 2020 was \$0.1 million compared to \$0.2 million for the three months ended September 30, 2019. Other income consisted primarily of interest earned on invested cash balances.

[Table of Contents](#)

Comparison of the nine months ended September 30, 2020 and 2019

The following table summarizes our results of operations for the nine months ended September 30, 2020 and 2019:

	Nine Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
Operating expenses:			
Research and development	\$ 42,158	\$ 37,403	\$ 4,755
General and administrative	14,611	8,741	5,870
Total operating expenses	56,769	46,144	10,625
Loss from operations	(56,769)	(46,144)	(10,625)
Other income (expense):			
Interest income and other income (expense), net	472	890	(418)
Net loss	\$ (56,297)	\$ (45,254)	\$ (11,043)

Research and development expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2020 and 2019:

	Nine Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
Personnel-related	\$ 12,546	\$ 9,602	\$ 2,944
Stock-based compensation	2,967	2,039	928
Preclinical and manufacturing	11,718	11,494	224
Facilities	7,099	7,024	75
Lab supplies	2,936	3,198	(262)
Consulting and professional services	2,494	1,760	734
Other	2,398	2,286	112
Total research and development expenses	\$ 42,158	\$ 37,403	\$ 4,755

Research and development expenses were \$42.2 million for the nine months ended September 30, 2020, compared to \$37.4 million for the nine months ended September 30, 2019. The increases in personnel-related costs and stock-based compensation costs of \$2.9 million and 0.9 million, respectively, were primarily due to increased headcount in our research and development function. The increase in consulting and professional services of \$0.7 million was primarily due to consulting services to develop and scale our manufacturing process.

General and administrative expenses

The following table summarizes our general and administrative expenses for the nine months ended September 30, 2020 and 2019:

	Nine Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
Personnel-related	\$ 5,555	\$ 3,121	\$ 2,434
Stock-based compensation	2,066	1,083	983
Professional and consultant fees	5,293	2,974	2,319
Facilities	1,120	850	270
Other	577	713	(136)
Total general and administrative expenses	\$ 14,611	\$ 8,741	\$ 5,870

General and administrative expenses were \$14.6 million for the nine months ended September 30, 2020, compared to \$8.7 million for the nine months ended September 30, 2019. The increases in personnel-related costs and stock-based compensation costs of \$2.4 million and \$1.0 million, respectively, were primarily a result of an increase in headcount in our general and administrative function.

Professional and consultant fees increased by \$2.3 million primarily due to professional fees relating to accounting, audit and legal services as well as costs associated with ongoing business activities and our preparations to operate as a public company.

[Table of Contents](#)

Other income and expense, net

Other income and expense, net for the nine months ended September 30, 2020 was \$0.5 million compared to \$0.9 million for the nine months ended September 30, 2019. Other income consisted primarily of interest earned on invested cash balances. The decrease in interest income from the nine months ended September 30, 2019 to the nine months ended September 30, 2020 was primarily due to lower invested balances and lower interest rates during 2020.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. We expect to incur significant expenses and operating losses for the foreseeable future as we support our continued research activities and development of our programs and platform. We have not yet commercialized any of our product candidates and we do not expect to generate revenue from sales of any product candidates for several years, if at all. To date, we have funded our operations with proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), the sales of convertible preferred stock and, most recently, with proceeds from the sale of common stock in our IPO. On June 16, 2020, we completed our IPO, pursuant to which we issued and sold 12,105,263 shares of our common stock, including 1,578,947 shares sold by us pursuant to the full exercise of the underwriters' option to purchase additional shares. We received net proceeds of \$210.7 million, after deducting underwriting discounts and commissions and other offering expenses. As of September 30, 2020, we had cash, cash equivalents and marketable securities of \$280.4 million.

Cash flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	Nine Months Ended September 30,	
	2020	2019
	(in thousands)	
Cash used in operating activities	\$ (51,777)	\$ (28,539)
Cash provided by (used in) investing activities	(225,547)	43,063
Cash provided by financing activities	320,798	9
Net increase in cash, cash equivalents and restricted cash	<u>\$ 43,474</u>	<u>\$ 14,533</u>

Operating activities

During the nine months ended September 30, 2020, operating activities used \$51.8 million of cash, primarily resulting from our net loss of \$56.3 million and changes in our operating assets and liabilities of \$3.0 million, both partially offset by non-cash charges of \$7.6 million. Net cash used by changes in our operating assets and liabilities for the nine months ended September 30, 2020 consisted primarily of a \$2.9 million increase in prepaid expenses and other current assets and a \$0.8 million decrease in deferred rent, partially offset by a decrease of \$0.4 million in tenant receivable.

During the nine months ended September 30, 2019, operating activities used \$28.5 million of cash, primarily resulting from our net loss of \$45.3 million, partially offset by non-cash charges of \$4.0 million and cash provided by changes in our operating assets and liabilities of \$12.7 million. Net cash provided by changes in our operating assets and liabilities for the nine months ended September 30, 2019 consisted primarily of a \$13.0 million increase in deferred rent and a \$2.2 million increase in accounts payable and accrued expenses and other current liabilities, both partially offset by an increase of \$1.3 million in tenant receivable and \$1.1 million in prepaid expenses and other current assets.

Changes in accounts payable, accrued expenses and other current liabilities and prepaid expenses in the periods were generally due to growth in our business and the timing of vendor invoicing and payments. Changes in deferred rent and tenant receivable primarily related to a tenant improvement allowance from our landlord.

Investing activities

During the nine months ended September 30, 2020, net cash used in investing activities was \$225.5 million, due to the purchases of marketable securities and property and equipment during the period. During the nine months ended September 30, 2019, net cash provided by investing activities was \$43.1 million, due primarily to net sales and maturities of marketable securities of \$61.9 million, partially offset by the acquisition of property and equipment during the period of \$18.8 million.

[Table of Contents](#)

Property and equipment purchases during the nine months ended September 30, 2020 and 2019 were primarily related to leasehold improvements and lab equipment for our facility in Cambridge, Massachusetts.

Financing activities

During the nine months ended September 30, 2020, net cash provided by financing activities was \$320.8 million, consisting primarily of proceeds from our IPO of common stock of \$213.9 million, net of underwriting discounts and commissions, and proceeds from the sale of our Series C preferred stock of \$108.9 million, partially offset by the payment of \$3.2 million of public offering costs. During the nine months ended September 30, 2019, net cash provided by financing activities was less than \$0.1 million, consisting of proceeds from the exercise of common stock options.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and initiate clinical trials for our product candidates in development. The timing and amount of our operating expenditures will depend largely on:

- the identification of additional research programs and additional product candidates;
- the scope, progress, costs and results of preclinical and clinical development for any product candidates we may develop;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidates we may develop for which we receive marketing approval;
- the costs and scope of the continued development of our non-viral gene therapy platform;
- the costs of satisfying any post-marketing requirements;
- the revenue, if any, received from commercial sales of product candidates we may develop for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting applications for patents, obtaining, maintaining, defending and enforcing our intellectual property rights and defending against any intellectual property-related claims, including claims of infringement, misappropriation or other violation of third-party intellectual property;
- the costs of operational, financial and management information systems and associated personnel;
- the associated costs in connection with any acquisition of in-licensed products, intellectual property and technologies; and
- the costs of operating as a public company.

We believe that our existing cash and cash equivalents, and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into 2023. We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong. We could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure 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 do not have any committed external source of funds. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. If we raise additional funds by issuing equity securities, our stockholders may experience dilution. Any future debt financing into which we enter would result in fixed payment obligations and may involve agreements that include grants of security interests on our assets and restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, granting liens over our assets, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. Any debt financing or additional equity that we raise may contain terms that could adversely affect the holdings or the rights of our common stockholders.

If we are unable to raise sufficient capital as and when needed, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate we may develop, or be unable to expand our operations or otherwise capitalize on our business opportunities. If we raise additional funds through collaborations or licensing arrangements with third parties, we may have to relinquish valuable rights to future revenue streams or product candidates or grant licenses on terms that may not be favorable to us.

[Table of Contents](#)

See “Risk Factors” for additional risks associated with our substantial capital requirements.

Contractual Obligations and Commitments

During the three months ended September 30, 2020, there were no material changes to our contractual obligations and commitments from those described under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Contractual Obligations and Commitments” in our final prospectus for our IPO filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on June 12, 2020.

Critical Accounting Policies and Significant Judgments and Estimates

We prepare our condensed consolidated financial statements in accordance with GAAP. The preparation of financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, costs and expenses and related disclosures. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ significantly from the estimates made by our management.

There have been no material changes to our critical accounting policies and estimates from those disclosed in our financial statements and the related notes and other financial information included in our final prospectus for our IPO filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on June 12, 2020.

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 in the rules and regulations of the Securities and Exchange Commission.

Recently Issued and Adopted Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our condensed consolidated financial statements included in this Quarterly Report.

Item 3. Quantitative and Qualitative Disclosures about Market Risks.

As of September 30, 2020, we had cash, cash equivalents, and marketable securities of \$280.4 million, which consisted of cash, money market funds, U.S. government securities, commercial paper and corporate securities. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, an immediate 10% change in market interest rates would not have a material effect on the fair market value of our investment portfolio. We are not currently exposed to significant market risk related to changes in foreign currency exchange rates. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, under the supervision and with the participation of our Principal Executive Officer (our Chief Executive Officer) and Principal Financial Officer (our Chief Financial Officer, Treasurer), has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2020. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or 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, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any disclosure 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, our Principal Executive Officer and Principal Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2020 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

Item 1A. Risk Factors.

Our business is subject to numerous risks. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Quarterly Report on Form 10-Q, or Quarterly Report, including our consolidated financial statements and the related notes thereto in evaluating our company. The risks described below are not the only risks facing our company. The occurrence of any of the following risks, or of additional risks and uncertainties not presently known to us or that we currently believe to be immaterial, could cause our business, prospects, operating results and financial condition to suffer materially.

Risks related to our financial position and need for additional capital

We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses over the next several years.

Since inception, we have incurred significant operating losses. Our net losses were \$61.3 million for the year ended December 31, 2019 and \$56.3 million for the nine months ended September 30, 2020. As of September 30, 2020, we had an accumulated deficit of \$164.7 million. To date, we have funded our operations with the proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), the sale of convertible preferred stock and, most recently, from the sale of common stock in our initial public offering, or IPO. We have devoted substantially all of our financial resources and efforts to research and development. We are still in the early stages of development of our product candidates, and we have not commenced or completed clinical development. We expect to continue to incur significant expenses and operating losses over the next several years. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue our current research programs and conduct additional research programs;
- advance any product candidates we identify into preclinical and clinical development;
- expand the capabilities of our proprietary non-viral gene therapy platform;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;

Table of Contents

- hire additional clinical, regulatory and scientific personnel;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates we may develop for which we may obtain regulatory approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development, future commercialization efforts and operations as a public company.

Even if we obtain regulatory approval of and are successful in commercializing one or more of any product candidates we may develop, we will continue to incur substantial research and development and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue.

We have never generated revenue from product sales and may never achieve or maintain profitability.

We have not initiated clinical development of any product candidate and expect that it will be many years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must succeed in developing, obtaining the necessary regulatory approvals for and eventually commercializing a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including:

- identifying product candidates and completing preclinical and clinical development of any product candidates we may identify;
- obtaining regulatory approval for any product candidates we may develop;
- manufacturing, marketing and selling any products for which we may obtain regulatory approval;
- achieving market acceptance of any product candidates we may develop for which we obtain regulatory approval as a viable treatment option; and
- satisfying any post-marketing requirements.

We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. We are currently only in the preclinical stage of our research programs. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or even continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we identify, continue the research and development of, initiate preclinical testing and clinical trials of and potentially seek marketing approval for any product candidates we may develop. In addition, if we obtain marketing approval for any product candidates we may develop, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed, on attractive terms or at all, we may be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

As of September 30, 2020, we had cash, cash equivalents and marketable securities of \$280.4 million. We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into 2023. However, we have based this estimate on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us. As a result, we could deplete our capital resources sooner than we currently expect and could be forced to seek additional funding sooner than planned.

Our future capital requirements will depend on many factors, including:

- the identification of additional research programs and additional product candidates;

Table of Contents

- the scope, progress, costs and results of preclinical and clinical development for any product candidates we may develop;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidates we may develop for which we receive marketing approval;
- the costs and scope of the continued development of our non-viral gene therapy platform;
- the costs of satisfying any post-marketing requirements;
- the revenue, if any, received from commercial sales of product candidates we may develop for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting applications for patents, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims, including claims of infringement, misappropriation or other violations of third-party intellectual property;
- the costs of operational, financial and management information systems and associated personnel;
- the associated costs in connection with any acquisition of in-licensed products, intellectual property and technologies; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully identify and develop product candidates and those are approved, we may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if at all, and such revenues may not be sufficient to sustain our operations. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize any product candidates. We cannot be certain that additional funding will be available on acceptable terms, or at all. We have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or other research and development initiatives. We could be required to seek collaborators for product candidates we may develop at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to product candidates we may develop in markets where we otherwise would seek to pursue development or commercialization ourselves.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate we may develop, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

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/or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, selling or licensing our assets, making capital expenditures, declaring dividends or encumbering our assets to secure future indebtedness.

If we raise additional funds through 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 grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other

[Table of Contents](#)

arrangements when needed or on terms acceptable to us, we would 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.

Our limited operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2016, and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, conducting research activities and filing and prosecuting patent applications. All of our research programs are still in the research or preclinical stage of development, and their risk of failure is high. We have not yet demonstrated our ability to initiate or complete any clinical trials, obtain marketing approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions our stockholders make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing genetic medicine products.

Our limited operating history, particularly in light of the rapidly evolving genetic medicine field, may make it difficult to evaluate our technology and industry and predict our future performance. Our limited history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early stage companies in rapidly evolving fields. If we do not address these risks successfully, our business will suffer.

In addition, as our business grows, we may encounter unforeseen expenses, restrictions, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with a research focus to a company capable of conducting development activities and then to a company supporting commercial activities. We may not be successful in such transitions.

Our ability to utilize our net operating loss carryforwards may be subject to limitations.

We have a history of cumulative losses and anticipate that we will continue to incur significant losses in the foreseeable future; thus, we do not know whether or when we will generate taxable income necessary to utilize our net operating losses, or NOLs, or research and development tax credit carryforwards. As of December 31, 2019, we had federal NOLs of \$91.9 million and state NOLs of \$90.6 million.

In general, under Sections 382 and 383 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, a corporation that undergoes an “ownership change,” generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a three-year period, is subject to limitations on its ability to utilize its pre-change NOLs and research and development tax credit carryforwards to offset future taxable income. We have not conducted a study to assess whether any such ownership changes have occurred. We may have experienced such ownership changes in the past and may experience such ownership changes in the future through subsequent changes in our stock ownership (which may be outside our control). As a result, if, and to the extent that, we earn net taxable income, our ability to use our pre-change NOLs and research and development tax credit carryforwards to offset such taxable income may be subject to limitations.

There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing NOLs could expire or otherwise become unavailable to offset future income tax liabilities. The Tax Cuts and Jobs Act, or the Tax Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

Risks related to discovery and development

We are very early in our development efforts. We have not identified any product candidates for IND-enabling studies or clinical development and as a result it will be years before we commercialize a product candidate, if ever. If we are unable to identify and advance product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts and have invested our research efforts to date in developing our platform. We have a portfolio of programs that are in early stages of preclinical development and have not identified any product candidates for IND-enabling studies or clinical development. We may never identify any product candidates or advance to clinical-stage development. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

[Table of Contents](#)

Commencing clinical trials in the United States is subject to acceptance by the U.S. Food and Drug Administration, or FDA, of an IND application and finalizing the trial design based on discussions with the FDA and other regulatory authorities. In the event that the FDA requires us to complete additional preclinical studies or we are required to satisfy other FDA requests prior to commencing clinical trials, the start of our first clinical trials may be delayed. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials or impose stricter approval conditions than we currently expect. There are equivalent processes and risks applicable to clinical trial applications in other countries, including countries in the European Union.

Commercialization of any product candidates we may develop will require preclinical and clinical development; regulatory and marketing approval in multiple jurisdictions, including by the FDA and the European Medicines Agency, or EMA; obtaining manufacturing supply, capacity and expertise; building of a commercial organization; and significant marketing efforts. The success of product candidates we may identify and develop will depend on many factors, including the following:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable;
- effective INDs or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for any product candidates we may develop;
- successful enrollment and completion of clinical trials, including under the FDA's current Good Clinical Practices, or GCPs, current Good Laboratory Practices, or cGLPs, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our future clinical programs that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended populations of any product candidates we may develop;
- receipt of marketing approvals from applicable regulatory authorities;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense and enforcement of patent, trademark, trade secret and other intellectual property protection or regulatory exclusivity for any product candidates we may develop;
- commercial launch of any product candidates we may develop, if approved, whether alone or in collaboration with others;
- acceptance of the benefits and use of our product candidates we may develop, including method of administration, if and when approved, by patients, the medical community and third-party payers;
- effective competition with other therapies;
- maintenance of a continued acceptable safety, tolerability and efficacy profile of any product candidates we may develop following approval; and
- establishment and maintenance of healthcare coverage and adequate reimbursement by payers.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates we may develop, which would materially harm our business. If we are unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing any product candidates we determine to develop on a timely basis, if at all.

The risk of failure for any product candidates we determine to develop is high. It is impossible to predict when or if any product candidate would prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of product candidates in humans. We have not yet begun or completed a clinical trial of any product candidate. Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses. Even if the clinical trials are successful, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application.

[Table of Contents](#)

Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our INDs and other regulatory filings. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of any product candidates. As a result, we cannot be sure that we will be able to submit INDs for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs will result in the FDA allowing clinical trials to begin. Furthermore, product candidates are subject to continued preclinical safety studies, which may be conducted concurrently with our clinical testing. The outcomes of these safety studies may delay the launch of or enrollment in future clinical trials and could impact our ability to continue to conduct our clinical trials.

Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. A failure of one or more clinical trials can occur at any stage of testing, which may result from a multitude of factors, including, but not limited to, flaws in trial design, dose selection issues, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits.

Identifying and qualifying patients to participate in clinical trials of any product candidates we may develop is critical to our success. We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

- perceived risks and benefits of novel genetic medicine-based approaches;
- size of the patient population, in particular for rare diseases, and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease or disorder under investigation;
- proximity and availability of clinical trial sites for prospective patients;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would harm our business, financial condition, results of operations and prospects.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CLROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board, or IRB, or independent ethics committee approval, or the equivalent review groups for sites outside the United States, at each clinical trial site;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- failure by us, any CLROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's GCPs;
- failure by physicians to adhere to delivery protocols leading to variable results;
- delays in the testing, validation, manufacturing and delivery of any product candidates we may develop to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;

Table of Contents

- occurrence of serious adverse events associated with a product candidate in development by another company, which are viewed to outweigh its potential benefits, and which may negatively impact the perception of our product due to a similarity in technology or approach;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the legal or regulatory regimes domestically or internationally related to patient rights and privacy; or
- lack of adequate funding to continue the clinical trial.

Any inability to successfully complete preclinical studies and clinical trials could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to any product candidates we may develop, we may need to conduct additional studies or trials to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize any product candidates we may develop or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize any product candidates we may develop and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of future clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with any product candidates we may develop, we may:

- be delayed in obtaining marketing approval for product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Genetic medicine is an emerging area of drug development that poses many scientific and other risks. We have only limited prior experience in genetic medicine research and manufacturing and no prior experience in genetic medicine clinical development. Our lack of experience for our genetic medicine programs may limit our ability to be successful or may delay our development efforts.

Genetic medicine is an emerging field of drug development with only a small number of genetic medicines having received FDA or EMA approval to date. Our genetic medicine research programs are still at an early stage, and there remain several areas of drug development risk, which pose particular uncertainty for our programs given the relatively limited development history of, and our limited prior experience with, genetic medicines. Translational science, manufacturing materials and processes, safety concerns, regulatory pathway and clinical trial design and execution all pose particular risk to our drug development activities. Furthermore, the medical community's understanding of the genetic causes of many diseases continues to evolve and further research may change the medical community's views on what therapies and approaches are most effective for addressing certain diseases.

As an organization, we have not previously conducted any IND-enabling studies or clinical trials, including any later stage or pivotal clinical trials. In pursuing our new technologies, we have begun to establish our own genetic medicine technical capabilities, but we will need to continue to expand those capabilities by either hiring internally or seeking assistance from outside service providers. Genetic medicine is an area of significant investment by biotechnology and pharmaceutical companies and there may be a scarcity of talent available to us in these areas. If we are not able to expand our genetic medicine capabilities, we may not be able to develop in the way we intend or desire any promising product candidates that emerge from our program or our other collaborative genetic medicine sponsored research programs, which would limit our prospects for future growth. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we may develop. Failure to commence or complete, or delays in, our clinical trials, could prevent us from or delay us in commercializing our product candidates.

We will need to build our internal and external capabilities in designing and executing a genetic medicine clinical trial. There are many known and unknown risks involved in translating preclinical development of gene therapies to clinical development, including

selecting appropriate endpoints and dosage levels for dosing humans based on preclinical data. Furthermore, our genetic medicine programs are initially targeting rare diseases with relatively small populations, which limits the pool of potential subjects for our genetic medicine clinical trials. If we are unable to initiate and conduct our genetic medicine clinical trials in a manner that satisfies our expectations or regulatory requirements, the value of our genetic medicine programs may be diminished.

Our non-viral gene therapy platform is based on novel technologies that are unproven, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval, if at all.

We have concentrated our research and development efforts on our non-viral gene therapy platform, and our future success depends on the successful development of our platform.

However, the technologies that comprise our platform are new and largely unproven. These technologies have not been clinically tested and the scientific evidence to support the feasibility of developing product candidates based on those technologies is both preliminary and limited. Successful development of product candidates by us will require solving a number of issues, including the expansion of our cell-targeted lipid nanoparticle delivery system, or ctLNP, delivery system to tissues beyond the liver and retina and obtaining expression levels sufficient to address or ameliorate each target disease or indication. There can be no assurance we will be successful in solving any or all of these issues. We have concentrated our research efforts to date on developing the components of our platform, and our future success is highly dependent on the successful development of our ceDNA constructs, our ctLNP delivery system and therapeutic applications of these technologies. We may decide to alter or abandon our initial programs as new data become available and we gain experience in developing our therapeutics. We cannot be sure that our technologies will yield satisfactory products that are safe and effective, scalable or profitable in any indication we pursue.

There can be no assurance that any development problems we experience in the future related to our non-viral gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from initiating or conducting clinical trials or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate may vary according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates, and even more so for product candidates intended to treat diseases for which there are approved therapies that may allow a normal life span as compared with those intended to treat serious and life threatening diseases for which there are no other treatment options available. Only a small number of non-viral gene therapies have successfully reached the clinical trial phase of development, limiting insight into the regulatory review process for this field of genetic medicine. Requirements by regulatory authorities for any product candidate we may develop may change in response to the availability of other therapies for the indication our product candidates are designed to treat or to issues observed in clinical trials of genetic medicines of other companies, even if using technology that differs from ours. We intend to pursue genetic medicine programs in both rare and prevalent diseases, some of which may have available approved therapies. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals in either the United States or the European Union for any product candidates we may develop or how long it will take to commercialize any product candidate that receives marketing approval.

If any product candidates we may develop cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

We have not evaluated any product candidates in human clinical trials. Moreover, there have been only a limited number of clinical trials involving the use of non-viral gene therapies and none involving ceDNA constructs or other technology similar to our technology. It is impossible to predict when or if any product candidates we may develop will prove safe in humans. In the genetic medicine field, there have been several significant adverse events from gene therapy treatments in the past, including reported cases of leukemia and death. There can be no assurance that our technologies will not cause undesirable side effects.

We use a ctLNP delivery system to deliver our ceDNA constructs. Lipid nanoparticles have been shown to induce necrosis in the liver at certain doses and induce infusion related reactions, as well as to initiate systemic inflammatory responses. While our ctLNPs are a new generation of LNP, there can be no assurance that our ctLNPs will not have undesired effects. Our ctLNPs could contribute, in whole or in part, to immune reactions, infusion reactions, complement reactions or antibody reactions. In addition, certain aspects of our non-viral gene therapies may induce immune reactions from the lipid as well as adverse reactions within liver pathways or degradation of the LNP into its component molecules or metabolites, any of which could lead to significant adverse events in one or more of our future clinical trials. Many of these types of side effects have been seen for LNPs. Once delivered to target cells, DNA-based payloads, such as those carried by our ceDNA constructs, may interact with host proteins or chromosomal DNA in the cell endosome, cytosol or nucleus.

Adeno-associated virus, or AAV, genomes have been shown in some cases to initiate intracellular immune activation, which can lead to transcriptional changes, and local tissue interferon responses, which may lead to immune infiltrates and tissue damage. AAV genetic material may also integrate into the host chromosome, which could contribute to modified cell function transformation. There may be uncertainty as to the underlying cause of any such adverse event, which would make it difficult to accurately predict side effects in future clinical trials and would result in significant delays in our programs.

If any product candidates we develop are associated with serious adverse events, undesirable side effects or unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations and prospects. Many product candidates that initially showed promise in early stage testing for treating cancer or other diseases have later been found to cause side effects that prevented further clinical development of the product candidates.

If in the future we are unable to demonstrate that such side effects were caused by factors others than our product candidates, the FDA, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates for any or all targeted indications. Even if we are able to demonstrate that any future serious adverse events are not product-related, and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any product candidate, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Regulatory approval of and/or demand for our potential products will depend in part on public acceptance of the use of genetic medicine for the prevention or treatment of human diseases. Safety issues that might arise in trials for gene therapies other than our own could adversely impact public attitudes towards our platform and product candidates notwithstanding that the gene therapies we are developing are non-viral.

There are a number of clinical trials of gene therapies ongoing. There is a potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry genetic material. Possible adverse side effects that may occur with treatment with gene therapy products include an immunologic reaction early after administration that could substantially limit the effectiveness of the treatment or represent safety risks for patients.

Any of these events could prevent us from achieving or maintaining market acceptance of any product candidates we may develop and could significantly harm our business, prospects, financial condition and results of operations.

The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.

We are in the early stage of research in the development of our platform and have not identified any product candidates or conducted any IND-enabling studies or any clinical trials. As a result, our belief in the capabilities of our platform is based on early research and preclinical studies. However, the results of preclinical studies may not be predictive of the results of preclinical studies or clinical trials, and the results of any early-stage clinical trials may not be predictive of the results of later clinical trials. In addition, initial success in clinical trials may not be indicative of results obtained when such trials are completed. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. Our future clinical trials may not ultimately be successful or support further clinical development of any product candidates we may develop. There is a high failure rate for product candidates proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving encouraging results in earlier studies. Any such setbacks in our clinical development could materially harm our business and results of operations.

We may not be successful in our efforts to identify, discover or develop potential product candidates.

The success of our business depends primarily upon our ability to identify, develop and commercialize product candidates based on our non-viral gene therapy platform. All of our product development programs are still in the research or preclinical stage of development. Our research programs may fail to identify potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates, our potential product candidates may be shown to have harmful side effects in preclinical *in vitro* experiments or animal model studies, they may not show promising signals of therapeutic effect in such experiments or studies or they may have other characteristics that may make the product candidates impractical to manufacture, unmarketable or unlikely to receive marketing approval.

[Table of Contents](#)

In addition, although we believe our platform will position us to rapidly expand our portfolio of programs beyond our current programs, we have not yet successfully developed any product candidate and our ability to expand our portfolio may never materialize. The process by which we identify and disclose product candidates may fail to yield product candidates for clinical development for a number of reasons, including those discussed in these risk factors and also:

- we may not be able to assemble sufficient resources to acquire or discover product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete or less attractive;
- potential product candidates we develop may nevertheless be covered by third parties' patents or other intellectual property rights;
- potential product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- potential product candidates may not be effective in treating their targeted diseases or disorders;
- the market for a potential product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a potential product candidate is too complex and difficult to navigate successfully or economically.

If we are unable to identify and discover suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price and could potentially cause us to cease operations.

The genetic medicine field is relatively new and evolving rapidly. We are focusing our research and development efforts on our non-viral gene therapy platform, but other gene therapy technologies may be discovered that provide significant advantages over our platform, which could materially harm our business.

To date, we have focused our efforts on the advancement of our non-viral gene therapy platform, which is designed to overcome the limitations of current viral gene therapy approaches. However, while these modalities have demonstrated their limitations, there are many companies that are developing new genetic medicines, including viral gene therapies, gene editing and messenger RNA, or mRNA. There can be no certainty that these companies will not develop genetic medicines that address some of these limitations and will be considered to have advantages over our non-viral gene therapy platform. For example, in December 2019, Dyno Therapeutics announced a new technique for AAV delivery, labeled BRAVE, that allows the researchers to engineer the virus shell to deliver the gene package to the exact cell type in the body they intend to treat. This new method may reduce concerns about off-target AAV delivery and make it a more attractive delivery system.

We may expend our limited resources to pursue a particular program, product candidate or indication and fail to capitalize on programs, product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and expect to focus on product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential, or we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable medicines. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects.

Clinical trial and product liability lawsuits against us could divert our resources, could cause us to incur substantial liabilities and could limit commercialization of any product candidates we may develop.

We will face an inherent risk of clinical trial and product liability exposure related to the testing of any product candidates we may develop in clinical trials, and we will face an even greater risk if we commercially sell any products that we may develop. While we currently have no product candidates in clinical trials or that have been approved for commercial sale, the future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates we may develop;

[Table of Contents](#)

- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any product candidates we may develop.

We will need to increase our insurance coverage if we commence clinical trials or if we commence commercialization of any product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Risks relating to manufacturing

The manufacture of genetic medicine products is complex and difficult and is subject to a number of scientific and technical risks, some of which are common to the manufacture of drugs and biologics and others of which are unique to the manufacture of gene therapies. We could experience manufacturing problems that result in delays in our development or commercialization programs.

Genetic medicine drug products are complex and difficult to manufacture. We have an established current Good Manufacturing Practices, or cGMP, -ready process at the 200-liter scale which we have successfully transferred to external contract development and manufacturing organizations, or CDMOs, to supply our gene therapies for IND-enabling preclinical studies and early clinical trials. We believe that we will be able to enter into arrangements with existing and/or additional CDMOs to provide commercial supply. We may also seek to eventually establish our own manufacturing facility for long-term commercial supply.

A number of factors common to the manufacturing of biologics and drugs could also cause production issues or interruptions for our gene therapies, including raw material or starting material variability in terms of quality, cell line viability, productivity or stability issues, shortages of any kind, shipping, distribution, storage and supply chain failures, growth media contamination, equipment malfunctions, operator errors, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, or acts of god that are beyond our or our contract manufacturer's control. It is often the case that early stage process development is conducted with materials that are not manufactured using cGMP starting materials, techniques or processes and which are not subject to the same level of analysis that would be required for clinical grade material. We may encounter difficulties in translating the manufacturing processes used to produce research grade materials to cGMP compliant processes, and any changes in the manufacturing process may affect the safety and efficacy profile of our product candidates.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage. Some of the raw materials that we anticipate will be required in our manufacturing process, such as Sf9 cells, are derived from biologic sources. Such raw materials may be difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of any product candidates we may develop could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations and prospects.

Our non-viral gene therapy platform is novel, and the combination of novel constructs with untested scaling may cause us to experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization programs, limit the supply of any product candidates we may develop or otherwise harm our business.

Our non-viral gene therapy platform is novel and the manufacture of products on the basis of our platform is untested at a large scale. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, insufficient inventory or potentially delay progression of our preclinical or clinical development of any product candidates we may develop. If we successfully develop product candidates, we may encounter problems achieving adequate quantities and quality that meet FDA, EMA or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. The ability to scale our manufacturing and maintain the manufacturing process at the same levels of quality and efficacy that we are currently manufacturing is yet to be tested. If we or our CDMOs are unable to scale our manufacturing at the same levels of quality and efficiency, we may not be able to supply the required number of doses for clinical trials or commercial supply, and our business could be harmed.

Manufacturing the ctLNP component of a potential product candidate may be complex and difficult, and we could experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization, limit the supply of any product candidates we may develop or otherwise harm our business.

Many product candidates we may develop will require the manufacture of the ctLNP component, which may require processing steps that are more complex than those required for current products that utilize LNPs. In order to manufacture ctLNPs that are specialized for a given platform program, we may need to add biologic ligands to existing LNPs. This process is challenging and may pose a risk to our ability to manufacture on a scale sufficient to meet clinical and commercial needs.

Testing of and changes to methods of product candidate manufacturing or formulation may result in additional costs or delay.

As product candidates proceed through preclinical studies to clinical trials towards potential approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are tested and then altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives.

An important part of the manufacturing of our potential product candidates is analytical testing. Analytical testing of gene therapies involves tests that are more numerous, more complex in scope and take a longer time to develop and to conduct as compared to traditional drugs. We and our CDMOs may need to expend considerable time and resources to develop assays and other analytical tests for our product candidates, including assays to assess the potency of our product candidates. Some assays may need to be outsourced to specialized testing laboratories. Even when assays are developed, they may need to be further tested, qualified and validated, which may take substantial time and resources. Because of the lagging nature of analytical testing, we may proceed with additional manufacturing and other development activities without having first fully characterized our manufactured materials. If the results of the testing fail to meet our expectations, we may need to delay or repeat certain manufacturing and development activities.

We may make changes to our manufacturing methods as part of our product development activities. Any such changes could cause any product candidates we may develop to perform differently and affect the results of clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, FDA notification or FDA approval. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue.

In addition, the FDA, the EMA, and other regulatory authorities may require us to submit samples of any lot of and approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA, or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

We currently depend on a small number of third-party suppliers for our drug substance and drug product, and we expect to continue to depend on third-party suppliers for materials used in the manufacture of any product candidates we may develop, and the loss of these third-party suppliers or their inability to supply us with adequate materials, particularly those raw materials that are in short supply, could harm our business.

We currently rely on a small number of third-party suppliers for our drug substance and drug product and expect to continue to rely on third-party suppliers for certain materials and components required for the production of any product candidates we may develop. Our dependence on these third-party suppliers and the challenges we may face in obtaining and maintaining adequate supplies of materials involve several risks, including limited control over pricing, availability and quality and delivery schedules. There is substantial demand and limited supply for certain of the raw materials used to manufacture genetic medicine products and these raw materials are usually sole-sourced, as there are a limited number of qualified suppliers. This limited supply, combined with any problems that may arise during the manufacturing process development, may create long lead times to manufacture or procure starting materials. The progress of our non-viral gene therapy platform is highly dependent on these suppliers providing us or our contract manufacturer with the necessary starting materials that meet our requirements in a timely manner. As a small company, our negotiation leverage is limited, and we are likely to get lower priority than our competitors that are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials that we require or satisfy our anticipated specifications and quality requirements.

Any supply interruption in limited or sole-sourced raw materials could materially harm our ability to manufacture any product candidates we may develop until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of any product candidates we may develop, including limiting supplies necessary for clinical trials and regulatory approvals, which would have a material adverse effect on our business.

Risks related to our dependence on third parties

We rely, and expect to continue to rely, on third parties to conduct some or all aspects of our product manufacturing, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our product manufacturing, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to many of these items, including CDMOs for the manufacturing of any product candidates we test in preclinical or clinical development, as well as contract research organizations, or CROs for the conduct of our animal testing and research. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical trials are conducted in accordance with the study plan and protocols.

Although we intend to design the clinical trials for any product candidates we may develop, CLROs will conduct some or all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less direct control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our preclinical studies and clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CLROs and other third parties do not perform preclinical studies and future clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of any product candidates we may develop may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates or our development programs may be materially and irreversibly harmed. If we are unable to rely on preclinical and clinical data collected by our CLROs and other third parties, we could be required to repeat, extend the duration of or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures.

If third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical studies and clinical trials required to support future IND submissions and approval of any product candidates we may develop.

We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including any contract manufacturers of any product candidates we may develop, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturer must supply all necessary documentation in support of a biologics license application, or BLA, on a timely basis and must adhere to the FDA's cGLP and cGMP regulations enforced through its facilities inspection program. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of any product candidates we may develop or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

[Table of Contents](#)

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be materially harmed.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms or at all if they are affiliated with our competitors;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities, particularly if they are under contract with our competitors;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in supply. An alternative manufacturer would need to be qualified through a BLA supplement which could result in further delay. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

We may from time to time be dependent on single-source suppliers for some of the components and materials used in, and the processes required to develop, our development candidates and investigational medicines.

We may from time to time depend on single-source suppliers for some of the components and materials used in, and manufacturing processes required to develop, our development candidates and investigational medicines. We cannot ensure that these suppliers or service providers will remain in business, have sufficient capacity or supply to meet our needs or that they will not be purchased by one of our competitors or another company that is not interested in continuing to work with us. Our use of single-source suppliers of raw materials, components, key processes and finished goods exposes us to several risks, including disruptions in supply, price increases or late deliveries. There are, in general, relatively few alternative sources of supply for substitute components. These vendors may be unable or unwilling to meet our future demands for our clinical trials or commercial sale. Establishing additional or replacement suppliers for these components, materials and processes could take a substantial amount of time and it may be difficult to establish replacement suppliers who meet regulatory requirements. Any disruption in supply from any single-source supplier or service provider could lead to supply delays or interruptions which would damage our business, financial condition, results of operations and prospects.

If we have to switch to a replacement supplier, the manufacture and delivery of our development candidates or investigational medicines could be interrupted for an extended period, which could adversely affect our business. Establishing additional or replacement suppliers for any of the components or processes used in our investigational medicines, if required, may not be accomplished quickly. If we are able to find a replacement supplier, the replacement supplier would need to be qualified and may require additional regulatory authority approval, which could result in further delay. While we seek to maintain adequate inventory of the single source components and materials used in our products, any interruption or delay in the supply of components or materials, or our inability to obtain components or materials from alternate sources at acceptable prices in a timely manner, could impair our ability to meet the demand for our investigational medicines.

If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state and local environmental, health, and safety laws, regulations and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research and product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws, regulations and permitting requirements. These current or future laws, regulations and permitting requirements may impair our research, development or production efforts. Failure to comply with these laws, regulations and permitting requirements also may result in substantial fines, penalties or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

We expect to rely on third parties to conduct, supervise and monitor IND-enabling studies and clinical studies, and if these third parties perform in an unsatisfactory manner, it may harm our business.

We expect to rely on CROs and CLROs and research and clinical trial sites to ensure our IND-enabling studies and clinical trials are conducted properly and on time. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs and CLROs' activities. Nevertheless, we will be responsible for ensuring that each of these studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs and CLROs does not relieve us of our regulatory responsibilities.

We and our CROs and CLROs will be required to comply with the FDA's GCPs for conducting, recording and reporting the results of IND-enabling studies and clinical trials to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. The FDA enforces these GCPs through periodic inspections of study sponsors, principal investigators and clinical trial sites. If we or our CROs or CLROs fail to comply with applicable GCPs, the preclinical and clinical data generated in our studies may be deemed unreliable and the FDA may require us to perform additional studies before approving any marketing applications. Upon inspection, the FDA may determine that our studies did not comply with GCPs.

Our CLROs and CROs are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs. These CLROs and CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our CROs or CLROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols or regulatory requirements, or for any other reasons, our studies may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize any product candidates we may develop. As a result, our financial results and commercial prospects would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

[Table of Contents](#)

We may enter into collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We may seek third-party collaborators for the research, development and commercialization of certain of the product candidates we may develop. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop pose numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay programs, preclinical studies or clinical trials, provide insufficient funding for programs, preclinical studies or clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with any product candidates we may develop if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators may be acquired by a third party having competitive products or different priorities;
- collaborators with marketing and distribution rights to one or more medicines may not commit sufficient resources to the marketing and distribution of such medicine or medicines;
- collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our medicines or any product candidates we may develop or that result in costly litigation or arbitration that diverts management attention and resources;
- we may lose certain valuable rights under certain circumstances, including if we undergo a change of control;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates we may develop; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished or terminated.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this Quarterly Report apply to the activities of our collaborators.

These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability

[Table of Contents](#)

to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of several factors. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

If conflicts arise between us and our collaborators or strategic partners, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.

If conflicts arise between our corporate or academic collaborators or strategic partners and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. Some of our academic collaborators are conducting multiple product development efforts within each area that is the subject of the collaboration with us. Our collaborators or strategic partners, however, may develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop that are the subject of these collaborations with us. Competing products, either developed by the collaborators or strategic partners or to which the collaborators or strategic partners have rights, may result in the withdrawal of partner support for our product candidates we may develop.

Some of our collaborators or strategic partners could also become our competitors in the future. Our collaborators or strategic partners could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, fail to devote sufficient resources to the development and commercialization of products, or merge with or be acquired by a third party who may do any of these things. Any of these developments could harm our product development efforts.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA, the EMA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization, reduce the scope of any sales or marketing activities, or increase our own expenditures on the development of the product candidate.

Risks related to commercialization

We face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to any product candidates that we may develop from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. 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 many of the disorders for which we are conducting 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. 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.

[Table of Contents](#)

There are numerous companies that are selling or developing genetic medicines, including in indications for which we may develop our non-viral gene therapies. These companies include viral gene therapy companies such as BioMarin Pharmaceuticals, Inc., Homology Medicines, Inc., Adverum Biotechnologies, Inc. and Hoffmann La Roche Ltd; gene editing companies such as Crispr Therapeutics AG, Intellia Therapeutics, Inc. and Editas Medicine, Inc.; and mRNA companies such as Moderna, Inc.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our product candidates or that would render any product candidates that we may develop obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than 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.

Many of the companies against which we are competing or against which we may compete in the future 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 and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Even if any product candidate that we may develop receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payers and others in the medical community necessary for commercial success.

If any product candidate we may develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payers and others in the medical community. Sales of medical products depend in part on the willingness of physicians to prescribe the treatment, which is likely to be based on a determination by these physicians that the products are safe, therapeutically effective and cost-effective. In addition, the inclusion or exclusion of products from treatment guidelines established by various physician groups and the viewpoints of influential physicians can affect the willingness of other physicians to prescribe the treatment. We cannot predict whether physicians, physicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that our product is safe, therapeutically effective and cost-effective as compared with competing treatments. Efforts to educate the medical community and third-party payers on the benefits of any product candidates we may develop may require significant resources and may not be successful. If any product candidates we may develop do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential advantages and limitations compared to alternative treatments;
- the effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments;
- the clinical indications for which the product is approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products, if approved, together with other medications.

The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our future product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

The initial target platforms in our pipeline are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payers, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect that coverage and reimbursement by third-party payers will be essential for most patients to be able to afford these treatments. Accordingly, sales of our future product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payers. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement by government authorities for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payers tend to follow CMS to a substantial degree. However, one payer's determination to provide coverage for a product does not assure that other payers will also provide coverage for the drug product. Further, a payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in the European Union may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as any product candidates we may develop. In many countries, particularly the countries of the European Union, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product, possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the United States, the reimbursement for any product candidates we may develop may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payers, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for any product candidates we may develop. We expect to experience pricing pressures in connection with the sale of any product candidates we may develop due to the trend toward managed healthcare, the increasing influence of certain third-party payers, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market. Recently there have been instances in which third-party payers have refused to reimburse treatments for patients for whom the treatment is indicated in the FDA-approved product label. Even if we are successful in obtaining FDA approvals to commercialize our product candidates, we cannot guarantee that we will be able to secure reimbursement for all patients for whom treatment with our product candidates is indicated.

In addition to CMS and private payers, professional organizations such as the American Medical Association, or the AMA, can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payers contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more product candidates for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

[Table of Contents](#)

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing any product candidates we may develop if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product for which we have obtained marketing approval, we will need to establish a sales, marketing and distribution organization, either ourselves or through collaborations or other arrangements with third parties.

In the future, we may build a sales and marketing infrastructure to market some of the product candidates we may develop if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. These efforts may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to educate adequate numbers of physicians on the benefits of any future products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payers;
- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are unable to establish our own sales, marketing and distribution capabilities and we enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute any product candidates we may develop or may be unable to do so on terms that are acceptable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing any product candidates we may develop.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Amendment, or the PPACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biological products.

There is a risk that any product candidates we may develop that are approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider any product candidates we may develop to be reference products for competing products, potentially creating

the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for nonbiological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

If the market opportunities for any product candidates we may develop are smaller than we believe they are, our potential revenues may be adversely affected, and our business may suffer. Because the target patient populations for many of the initial product candidates we may develop are small, we must be able to successfully identify patients and achieve a significant market share to maintain profitability and growth.

We are focusing our initial research and product development on treatments for rare genetically defined diseases; as a result, the relevant patient population may be small. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with product candidates we may develop, are based on estimates. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. The number of patients in the United States, Europe and elsewhere may turn out to be lower than expected, and patients may not be amenable to treatment with our product candidates we may develop, or may become increasingly difficult to identify or gain access to, all of which would adversely affect our business, financial condition, results of operations and prospects. Additionally, because of the potential that any product candidates we may develop could cure a target disease, we may not receive recurring revenues from patients and may deplete the patient population prevalence through curative therapy.

Risks related to our intellectual property

Although we own and license a number of pending patent applications which have not yet issued as patents, we do not currently own or exclusively in-license any issued patents relating to any product candidates we may develop or technology, including with respect to our ceDNA constructs, ctLNP delivery system and manufacturing process. If we or our licensors are unable to obtain, maintain and defend patent and other intellectual property protection for our product candidates and technology, or if the scope of the patent or other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully develop and commercialize any product candidates we may develop or our technology may be adversely affected due to such competition.

Our success depends in large part on our and our licensors' ability to obtain and maintain patent and other intellectual property protection in the United States and other jurisdictions with respect to any product candidates we may develop and our technology, including our ceDNA constructs, ctLNP delivery system, manufacturing processes and their respective components, formulations, combination therapies, methods of treatment, processes and development that are important to our business, as well as successfully defending these patents and other intellectual property against third-party challenges. We and our licensors have sought, and will seek, to protect our proprietary position by filing patent applications in the United States and abroad related to certain technologies and our platform that are important to our business. However, our patent portfolio is at an early stage and we currently do not own or exclusively license any issued patents in any jurisdiction. Moreover, there can be no assurance as to whether or when our patent applications will issue as granted patents. Our ability to stop third parties from making, using, selling, marketing, offering to sell, importing and commercializing any product candidates we may develop and our technology is dependent upon the extent to which we have rights under valid and enforceable patents and other intellectual property that cover our platform and technology. If we are unable to secure, maintain, defend and enforce patents and other intellectual property with respect to any product candidates we may develop and technology, it would have a material adverse effect on our business, financial condition, results of operations and prospects.

We own certain patent applications, and exclusively in-license from University of Massachusetts as represented by and solely on behalf of its Medical School, or UMass, and Voyager Therapeutics, Inc. certain other patent applications, which cover our ceDNA platform structure, use and/or function, our ctLNP platform and its use, and ceDNA manufacturing processes, as applicable. Our pending Patent Cooperation Treaty, or PCT, patent applications are not eligible to become issued patents until, among other things, we file a national stage patent application within 30 to 32 months, depending on the jurisdiction, from such application's priority date in the jurisdictions in which we are seeking patent protection. Similarly, our pending provisional patent applications are not eligible to become issued patents until, among other things, we file a non-provisional patent application within 12 months of such provisional patent application's filing date. If we do not timely file such national stage patent applications or non-provisional patent applications, we may lose our priority date with respect to such PCT or provisional patent applications, respectively, and any patent protection on the inventions disclosed in such PCT or provisional patent applications, respectively. While we and our licensors intend to timely file national stage and non-provisional patent applications relating to our PCT and provisional patent applications, respectively, we cannot predict whether any such patent applications will result in the issuance of patents. If we or our licensors do not successfully obtain issued patents, or, if the scope of any patent protection we or our licensors obtain is not sufficiently broad, we will be unable to prevent others from using any product candidates we may develop or our technology or from developing or commercializing technology and products similar or identical to ours or other competing products and technologies. Any failure to obtain or maintain

[Table of Contents](#)

patent protection with respect to our ceDNA constructs, ctLNP delivery system, manufacturing processes or our other product candidates and technology would have a material adverse effect on our business, financial condition, results of operations and prospects.

The patent prosecution process is expensive, time-consuming and complex, and we and our licensors may not be able to file, prosecute, maintain, defend, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. We and our licensors may not be able to obtain, maintain or defend patents and patent applications due to the subject matter claimed in such patents and patent applications being in the public domain. For example, in some cases, the work of certain academic researchers in the genetic medicine field has entered or will enter the public domain, which may compromise our and our licensors' ability to obtain patent protection for certain inventions related to or building upon such prior work. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. Consequently, we would not be able to prevent any third party from using any of our technology that is in the public domain to compete with our product candidates.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of patent rights are highly uncertain. Our pending and future owned and licensed patent applications may not result in patents being issued which protect our technology or product candidates, effectively prevent others from commercializing competitive technologies and product or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all, and even if such patent applications do issue as patents, they may not issue in a form, or with a scope of claims, that will provide us with any meaningful protection, prevent others from competing with us or otherwise provide us with any competitive advantage. In addition, the scope of claims of an issued patent can be reinterpreted after issuance, and changes in either the patent laws or interpretation of the patent laws in the United States and other jurisdictions may diminish the value of our patent rights or narrow the scope of our patent protection. Furthermore, our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

Third parties have developed technologies that may be related or competitive to our own technologies and product candidates and may have filed or may file patent applications, or may have obtained issued patents, claiming inventions that may overlap or conflict with those claimed in our owned or licensed patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to our current and future product candidates and technology. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know for certain whether the inventors of our owned or licensed patents and patent applications were the first to make the inventions claimed in any owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. If a third party can establish that we or our licensors were not the first to make or the first to file for patent protection of such inventions, our owned or licensed patent applications may not issue as patents and even if issued, may be challenged and invalidated or ruled unenforceable.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and other jurisdictions. For example, we may be subject to a third-party submission of prior art to the United States Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of our owned or licensed patents. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of our owned or licensed pending patent applications. We may become involved in opposition, derivation, re-examination, *inter partes* review, post-grant review or interference proceedings and similar proceedings in foreign jurisdictions (for example, opposition proceedings) challenging our owned or licensed patent rights. In addition, a third party may claim that our owned or licensed patent rights are invalid or unenforceable in a litigation. An adverse result in any litigation or patent office proceeding could put one or more of our owned or licensed patents at risk of being invalidated, ruled unenforceable or interpreted narrowly and could allow third parties to commercialize products identical or similar to any product candidates we may develop and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge priority of invention or other features of patentability. Such challenges and proceedings may result in loss of patent rights, exclusivity, freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and any product candidates we may develop. Such challenges and proceedings may also result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. Moreover, there could be public announcements of the results of hearings, motions or other interim proceedings or developments related to such challenges and proceedings 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.

[Table of Contents](#)

Furthermore, patents have a limited lifespan. In the United States, the expiration of a patent is generally 20 years from the earliest date of filing of the first non-provisional patent application to which the patent claims priority. Patent term adjustments and extensions may be available; however, the overall term of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent and other intellectual property rights may not provide us with sufficient rights to exclude others from commercializing products similar or identical to our technology and any product candidates we may develop. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

Our rights to develop and commercialize any product candidates are subject, in part, to the terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under our current or future intellectual property license agreements or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose intellectual property rights that are important to our business.

We are reliant upon licenses from third parties for certain patent and other intellectual property rights that are important or necessary to the development of our technology and product candidates. For example, we rely on a license from the National Institutes of Health, or NIH, and the Association Institut de Myologie, Universite Pierre et Marie Curie, Centre National de la Recherche Scientifique and Inserm Transfert SA, which we refer to as the French Institutions, pursuant to which we have been granted a non-exclusive, worldwide, royalty-bearing license to certain patent rights related to our ceDNA construct, to make and have made, research and have researched, use and have used, sell and have sold, offer to sell and to import products for the treatment, prevention or palliation of any human disease, disorder or condition. In addition, we rely on a license from UMass pursuant to which we have been granted an exclusive, worldwide, royalty-bearing license to certain patent rights related to our ceDNA construct to research, develop, manufacture, have manufactured, use, offer for sale, sell and import products in the treatment, prevention or palliation of any human disease, disorder or condition. Our existing license agreements, including our license agreements with NIH and UMass, impose, and we expect that future license agreements will impose, specified diligence, milestone payment, royalty, commercialization, development and other obligations on us and require us to meet development timelines, or to exercise diligent or commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses. We may enter into additional license agreements in the future.

Furthermore, the licensors of our license agreements have the right to terminate the agreement if we materially breach the agreement and fail to cure such breach within a specified period or in the event we undergo certain bankruptcy events. In spite of our best efforts, our current or any future licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements. If our license agreements are terminated, we may lose our rights to develop and commercialize any product candidates we may develop and technology, lose patent protection any product candidates we may develop and our technology, experience significant delays in the development and commercialization of our product candidates and technology and incur liability for damages. If these in-licenses are terminated, or if the underlying intellectual property fails to provide the intended exclusivity, our competitors or other third parties could have the freedom to seek regulatory approval of, and to market, products and technologies identical or competitive to ours and we may be required to cease our development and commercialization of certain of our product candidates and technology. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with any product candidates we may develop and our technology. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- our or our licensors' ability to obtain, maintain and defend intellectual property and to enforce intellectual property rights against third parties;
- the extent to which our technology, product candidates and processes infringe, misappropriate or otherwise violate the intellectual property of the licensor that is not subject to the license agreement;
- the sublicensing of patent and other intellectual property rights under our license agreements;
- our diligence, development, regulatory, commercialization, financial or other obligations under the license agreement and what activities satisfy those diligence obligations;

[Table of Contents](#)

- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, our license agreements are, and future license agreements are likely to be, complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our diligence, development, regulatory, commercialization, financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If disputes over intellectual property that we have licensed or any other dispute described above related to our license agreements prevent or impair our ability to maintain our current license agreements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates and technology. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our license agreement with NIH is, and other license agreements we may enter into in the future may be, non-exclusive. Accordingly, third parties may also obtain non-exclusive licenses from such licensors, including NIH, with respect to the intellectual property licensed to us under such license agreements, including our NIH license agreement. Accordingly, our NIH license agreement does not, and other license agreements may not, provide us with exclusive rights to use such licensed patent and other intellectual property rights, or may not provide us with exclusive rights to use such patent and other intellectual property rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and any product candidates we may develop in the future.

Moreover, some of our in-licensed patent and other intellectual property rights are, and may in the future be, subject to third party interests such as co-ownership. If we choose not to or are unable to obtain an exclusive license to such third-party co-owners' interest, in such patent and other intellectual property rights, such third-party co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. We or our licensors may need the cooperation of any such co-owners of our licensed patent and other intellectual property rights in order to enforce them against third parties, and such cooperation may not be provided to us or our licensors.

Additionally, we do not have complete control over the preparation, filing, prosecution, maintenance, enforcement and defense of patents and patent applications that we license from third parties. For example, pursuant to each of our intellectual property licenses with NIH and UMass, our licensors retain control of preparation, filing, prosecution and maintenance, and, in certain circumstances, enforcement and defense of their patents and patent applications. It is possible that our licensors' filing, prosecution and maintenance of the licensed patents and patent applications, enforcement of patents against infringers or defense of such patents against challenges of validity or claims of enforceability may be less vigorous than if we had conducted them ourselves, and accordingly, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to file, prosecute, maintain, enforce and defend such patents and patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our technology and any product candidates we may develop that are the subject of such licensed rights could be adversely affected and we may not be able to prevent competitors or other third parties from making, using and selling competing products. Any of these events could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

Furthermore, our owned and in-licensed patent rights may be subject to a reservation of rights by one or more third parties. For example, inventions contained within some of our in-licensed patent rights may be made using U.S. government funding. When new technologies are developed with government funding, in order to secure ownership of patent rights related to the technologies, the recipient of such funding is required to comply with certain government regulations, including timely disclosing the inventions claimed in such patent rights to the U.S. government and timely electing title to such inventions. We rely on our licensors to ensure compliance with applicable obligations arising from such funding, including such timely disclosure and election of title. The failure of our licensors to meet their obligations may lead to a loss of rights or the unenforceability of relevant patents or patent applications. In addition, the U.S. government has certain rights in such in-licensed patent rights, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology, which may include our confidential information, to third parties and to exercise march-in rights to use or allow third parties to use the technology we have licensed that was developed using U.S. government funding. The U.S. government may exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such in-licensed U.S. government-funded inventions may be subject to certain requirements to manufacture any product candidates we may develop embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations and prospects significantly.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, maintaining, enforcing and defending patents and other intellectual property rights on our technology and any product candidates we may develop in all jurisdictions throughout the world would be prohibitively expensive, and accordingly, our intellectual property rights in some jurisdictions outside the United States could be less extensive than those in the United States. In some cases, we or our licensors may not be able to obtain patent or other intellectual property protection for certain technology and product candidates outside the United States. In addition, the laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we and our licensors may not be able to obtain issued patents or other intellectual property rights covering any product candidates we may develop and our technology in all jurisdictions outside the United States and, as a result, may not be able to prevent third parties from practicing our and our licensors' inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Third parties may use our technologies in jurisdictions where we and our licensors have not pursued and obtained patent or other intellectual property protection to develop their own products and, further, may export otherwise infringing, misappropriating or violating products to territories where we have patent or other intellectual property protection, but enforcement is not as strong as that in the United States. These products may compete with any product candidates we may develop and our technology and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Additionally, many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain jurisdictions, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement, misappropriation or other violation of our patent and other intellectual property rights or marketing of competing products in violation of our intellectual property rights generally. For example, an April 2019 report from the Office of the United States Trade Representative identified a number of countries, including China, Russia, Argentina, Chile and India, where challenges to the procurement and enforcement of patent rights have been reported. Proceedings to enforce our or our licensors' patent and other intellectual property rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patent and other intellectual property rights at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We or our licensors may not prevail in any lawsuits that we or our licensors initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many jurisdictions have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many jurisdictions limit the enforceability of patents against government agencies or government contractors. In these jurisdictions, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or patent applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned or licensed patent rights. We rely on our outside counsel and other professionals or our licensing partners to pay these fees due to the USPTO and non-U.S. government patent agencies. The USPTO and various non-U.S. government patent agencies also require compliance with several procedural, documentary and other similar provisions during the patent application process. We rely on our outside counsel and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. Moreover, complications due to the COVID-19 pandemic may result in inadvertent lapse due to, for example, unexpected closures of the USPTO or foreign patent offices, delays in delivery of notifications relating to deadlines, or failure to timely and/or properly obtain signatures on necessary documents. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment, loss of priority or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

We may not be successful in obtaining necessary rights to product candidates we may develop through acquisitions and in-licenses.

We currently have rights to certain intellectual property through licenses from third parties. Because our programs may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these intellectual property rights. In addition, with respect to any patent or other intellectual property rights that we co-own with third parties, we may require exclusive licenses to such co-owners' interest in such patent or other intellectual property rights. However, we may be unable to secure such licenses or otherwise acquire or in-license any intellectual property rights related to compositions, methods of use, processes or other components from third parties that we identify as necessary for any product candidates we may develop and our technology on commercially reasonable terms, or at all. Even if we are able to in-license any such necessary intellectual property, it could be on non-exclusive terms, thereby giving our competitors and other third parties access to the same intellectual property licensed to us, and the applicable licensors could require us to make substantial licensing and royalty payments. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to third parties, potentially blocking our ability to pursue our research program and develop and commercialize our product candidates.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have licensed, we may be required to expend significant time and resources to redesign any product candidates we may develop or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Issued patents covering any product candidates we may develop could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.

Our owned and licensed patent rights may be subject to priority, validity, inventorship and enforceability disputes. If we or our licensors are unsuccessful in any of these proceedings, such patent rights may be narrowed, invalidated or held unenforceable, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or we may be required to cease the development, manufacture and commercialization of one or more of our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If one of our licensing partners, one of our co-owners, we or our licensor's other licensees initiate legal proceedings against a third party to enforce a patent covering any of any product candidates we may develop or our technology, the defendant could counterclaim that the patent covering the product candidate or technology is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of utility, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, interference proceedings, derivation proceedings, post grant review, *inter partes* review and equivalent proceedings such as opposition, invalidation and revocation proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover any product candidates we may develop or our technology or prevent third parties from competing with any product candidates we may develop or our technology. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates or technology. Such a loss of patent protection could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any

[Table of Contents](#)

other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, contractors and other parties who have access to such technology and processes. However, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees and consultants who are parties to these agreements breach or violate the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets and third parties could use our trade secrets to compete with any product candidates we may develop and our technology. Additionally, we cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems; however, such systems and security measures may be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors or other third parties. Competitors or third parties could purchase any product candidates we may develop or our technology and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our intellectual property rights or develop their own competitive technologies that fall outside the scope of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products, our business, financial condition, results of operations and prospects could be materially and adversely affected.

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 could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or be threatened with, adversarial proceedings or litigation in which third parties may assert infringement, misappropriation or other violation claims against us, alleging that any product candidates we may develop, manufacturing methods, formulations or administration methods are covered by their patents. Given the vast number of patents and other intellectual property in our field of technology, we cannot be certain or guarantee that we do not infringe, misappropriate or otherwise violate patents or other intellectual property. Other companies and institutions have filed, and continue to file, patent applications that may be related to our technology and, more broadly, to gene therapy and related manufacturing methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. If a patent holder believes the manufacture, use, sale or importation of any product candidates we may develop or our technology infringes its patent, the patent holder may sue us even if we have licensed other patent rights for our technology.

It is also possible that we have failed to identify relevant third-party patents or applications. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use, sale or importation of any product candidates we may develop or our technology and we may not be aware of such patents. Furthermore, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States may remain confidential until a patent issues. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to any product candidates we may develop and our technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, any product candidates we may develop or the use of any product candidates we may develop.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these

third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize any product candidates we may develop or any other of our 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 valid and enforceable intellectual property rights, 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. We 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 monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing any product candidates we may develop or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

Intellectual property litigation or other proceedings could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Competitors may challenge the validity and enforceability of our patent rights or those of our licensing partners, infringe, misappropriate or otherwise violate our or our licensors' patent and other intellectual property rights, or we may be required to defend against claims of infringement, misappropriation or other violation. Litigation and other proceedings in connection with any of the foregoing claims can be unpredictable, expensive and time consuming. Even if resolved in our favor, litigation or other proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our scientific, technical and management personnel from their normal responsibilities. In addition, there could 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. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors or other third parties may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace and could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or be required to obtain licenses to such intellectual property rights, which may not be available on commercially reasonable terms or at all. An inability to incorporate such intellectual property rights would harm our business and may prevent us from successfully commercializing any product candidates we may develop or at all. In addition, we may lose personnel as a result of such claims and any such litigation or the threat thereof may adversely affect our ability to hire employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize any product candidates we may develop and our technology, which would have a material adverse effect on our business, results of operations, financial condition and prospects. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to our scientific and management personnel.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Furthermore, individuals executing

agreements with us may have pre-existing or competing obligations to a third party, such as an academic institution, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property that we own may have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, we or our licensors may in the future be subject to claims by former employees, consultants or other third parties asserting an ownership right in our owned or licensed patent rights. An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar technology and therapeutics, without payment to us, or could limit the duration of the patent protection covering our technology and any product candidates we may develop. Such challenges may also result in our inability to develop, manufacture or commercialize our technology and product candidates without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our owned or licensed patent rights are threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future technology and product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Changes in patent law in the United States or worldwide could diminish the value of patents in general, thereby impairing our ability to protect any product candidates we may develop and our technology.

Changes in either the patent laws or interpretation of patent laws in the United States and worldwide, including patent reform legislation such as the Leahy-Smith America Invents Act, or the Leahy-Smith Act, could increase the uncertainties and costs surrounding the prosecution of any owned or in-licensed patent applications and the maintenance, enforcement or defense of any current in-licensed issued patents and issued patents we may own or in-license in the future. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, and enable third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent at USPTO-administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first-to-file system in which, assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our in-licensed issued patents and issued patents we may own or in-license in the future, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. As one example, in the case *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable simply because they have been isolated from surrounding material. Moreover, in 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to patent-ineligible subject matter. Accordingly, in view of the guidance memo, there can be no assurance that claims in our patent rights covering any product candidates we may develop or our technology will be held by the USPTO or equivalent foreign patent offices or by courts in the United States or in foreign jurisdictions to cover patentable subject matter. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop and our technology, one or more of our U.S. patents that we license or may own in the future may be eligible for limited patent term extension under Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved product, a method for using it or a method for manufacturing it may be extended. The application for the

extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims challenging the inventorship or ownership of our patent and other intellectual property rights.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patent rights, trade secrets or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates or technology. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patent rights, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of or right to use intellectual property that is important to any product candidates we may develop or our technology. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

We have filed trademark applications with the USPTO for the mark "Generation Bio" and the Generation Bio logo. Our current and future trademark applications in the United States and other foreign jurisdictions may not be allowed or may be subsequently opposed. Once filed and registered, our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. As a means to enforce our trademark rights and prevent infringement, we may be required to file trademark claims against third parties or initiate trademark opposition proceedings. This can be expensive and time-consuming, particularly for a company of our size. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations or prospects.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to any product candidates we may develop but that are not covered by the intellectual property, including the claims of the patents, that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing, misappropriating or otherwise violating our owned or licensed intellectual property rights;
- it is possible that our or our licensors' current or future pending patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by third parties;

[Table of Contents](#)

- third parties might conduct research and development activities in jurisdictions where we do not have patent or other intellectual property rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor or other third party will discover our trade secrets or that our trade secrets will be misappropriated or disclosed.

Because we currently rely on certain third parties to manufacture all or part of our drug product and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our product engine and pipeline, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements and other similar agreements with our collaborators, advisors, employees, consultants and contractors prior to beginning research or disclosing any proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors or other third parties, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's or other third party's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

Risks related to regulatory approval and other legal compliance matters

Regulatory requirements governing genetic medicine products, and in particular any novel gene therapy products we may develop, have changed frequently and may continue to change in the future.

Regulatory requirements governing gene and cell therapy products, and in particular any novel gene therapy products we may develop, have changed frequently and may continue to change in the future. We are aware of a limited number of gene therapy products that have received marketing authorization from the FDA and EMA. Even with respect to more established products in the gene therapy field, the regulatory landscape is still developing. For example, the FDA has established the Office of Tissues and Advanced Therapies (formerly the Office of Cellular, Tissue and Gene Therapies) within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, also are potentially subject to review by the Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC; however, the NIH announced that the RAC will soon only publicly review clinical trials if the trials cannot be evaluated by standard oversight bodies and pose unusual risks.

The same applies in the European Union. The EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety and efficacy of advanced-therapy medicinal products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the Committee for Medicinal Products for Human Use, or CHMP, before CHMP adopts its final opinion. In the European Union, the development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant European Union guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any product candidates we may develop, but that remains uncertain at this point.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of any product candidates we may develop or lead to significant post-approval limitations or restrictions. As we advance any product candidates we may develop, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of these product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

Although the FDA decides whether individual genetic medicine protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution to conduct a clinical trial, that institution's institutional biosafety committee, or IBC, as well as its IRB would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of genetic medicine products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any product candidates we may develop. Similarly, the EMA may issue new guidelines concerning the development and marketing authorization for genetic medicine products and require that we comply with these new guidelines.

As we are initially seeking to identify and develop product candidates to treat diseases using novel technologies, there is heightened risk that the FDA, the EMA or other regulatory authority may not consider the clinical trial endpoints that we propose to provide clinically meaningful results. Even if the endpoints are deemed clinically meaningful, we may not achieve these endpoints to a degree of statistical significance, particularly because many of the diseases we are targeting with our platform have small patient populations, making development of large and rigorous clinical trials more difficult.

Adverse developments in post-marketing experience or in clinical trials conducted by others of gene therapy products or cell therapy products may cause the FDA, the EMA, and other regulatory bodies to revise the requirements for development or approval of any product candidates we may develop or limit the use of products utilizing non-viral gene therapy technologies, either of which could materially harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as the product candidates we may develop can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing non-viral gene therapy technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays or other impediments to our research programs or the commercialization of resulting products.

In addition, ethical, social and legal concerns about genetic medicine, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that any product candidates we may develop are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of any product candidates we may develop under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

As we advance any product candidates we may develop through clinical development, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of any product candidates we may develop or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue.

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of any product candidates we may develop. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, product candidates we may develop, and our ability to generate revenue will be materially impaired.

Any product candidates we may develop and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate we may develop will prevent us from commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CLROs to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each

therapeutic indication to establish the biologic product candidate's safety, purity and potency. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we may develop may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities, or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved medicine not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact public perception of our future product candidates.

Our potential therapeutic products involve introducing genetic material into patients' cells. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical or immoral, and, consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. For example, in 2003, trials using early versions of murine gamma-retroviral vectors, which integrate with, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although our delivery system is non-viral, any product candidates we may develop may be associated with such viral delivery systems as a gene therapy platform. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical trials. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the European Union and many other foreign jurisdictions, we or our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Political and socioeconomic factors can adversely affect our business. For example, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the

[Table of Contents](#)

United Kingdom left the European Union on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020 (extendable up to two years). Discussions between the United Kingdom and the European Union have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult. To date, only an outline of a trade agreement has been reached. Much remains open but the Prime Minister has indicated that the United Kingdom will not seek to extend the transitional period beyond the end of 2020. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption. The Prime Minister has also indicated that the United Kingdom will not accept high regulatory alignment with the European Union.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for any product candidates we may develop, which could significantly and materially harm our business.

Fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of any product candidates we may develop.

If any product candidate we may develop is intended for the treatment of a serious or life-threatening condition and the product candidate demonstrates the potential to address unmet medical need for this condition, the sponsor may apply for FDA fast track designation. However, a fast track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive fast track designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

Breakthrough or RMAT therapy designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.

If any product candidate we may develop is intended, either alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development, the sponsor may apply for FDA breakthrough designation or a regenerative medicine advanced therapy, or RMAT, designation. However, neither a breakthrough designation nor an RMAT designation ensures that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive breakthrough or RMAT designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw breakthrough or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Neither breakthrough nor RMAT designation alone guarantees qualification for the FDA's priority review procedures.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.

If the FDA determines that a product candidate we may develop offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for any product candidates we may develop. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate we may develop is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

We may not be able to obtain orphan drug exclusivity for any product candidates we may develop, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

[Table of Contents](#)

In order for the FDA to grant orphan drug exclusivity to one of our products, the agency must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet this standard. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. In particular, the concept of what constitutes the “same drug” for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA has issued recent draft guidance suggesting that it would not consider two genetic medicine products to be different drugs solely based on minor differences in the transgenes or vectors. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

In 2017, the Congress passed the FDA Reauthorization Act of 2017, or the FDARA. FDARA, among other things, codified the FDA’s pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate we may develop or the approval may be for a more narrow indication than we expect.

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if any product candidates we may develop demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical trials and the review process. Regulatory agencies also may approve a product candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of any product candidates we may develop. For example, our development of any product candidates for pediatric use is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

Even if we, or any collaborators we may have, obtain marketing approvals for any product candidates we may develop, the terms of approvals and ongoing regulation of our products could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such medicine, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The FDA typically advises that patients treated with genetic medicine undergo follow-up observations for potential adverse events for a 15-year period. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine.

Accordingly, assuming we, or any collaborators we may have, receive marketing approval for one or more product candidates we may develop, we, and such collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we and such

[Table of Contents](#)

collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition and prospects.

If we fail to comply with applicable regulatory requirements following approval of any product candidates we may develop, a regulatory agency may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or supplements to a BLA submitted by us;
- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we may develop and generate revenues.

Any product candidate we may develop for which we obtain marketing approval could be subject to restrictions or withdrawal from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our medicines, when and if any of them are approved.

The FDA and other regulatory agencies closely regulate the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other regulatory agencies impose stringent restrictions on manufacturers' communications regarding off-label use, and if we do not market our medicines for their approved indications, we may be subject to enforcement action for off-label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice. Violation of the Federal Food, Drug, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown problems with our medicines, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such medicines, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a medicine;
- restrictions on the distribution or use of a medicine;
- requirements to conduct post-marketing clinical trials;
- receipt of warning or untitled letters;
- withdrawal of the medicines from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of medicines;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- suspension of any ongoing clinical trials;
- refusal to permit the import or export of our medicines;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we develop and adversely affect our business, financial condition, results of operations and prospects.

Additionally, if any product candidates we may develop receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to healthcare practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;

Table of Contents

- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

We are affected by the political environment and changes that may be made to regulatory regimes. The efforts of the current presidential administration to pursue regulatory reform may limit the FDA's ability to engage in oversight and implementation activities in the normal course, and that could negatively impact our business.

The current presidential administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance and review and approval of marketing applications. On January 30, 2017, the president issued an executive order, applicable to all executive agencies, including the FDA, that required that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This executive order includes a budget neutrality provision that required the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the executive order requires agencies to identify regulations to offset any incremental cost of a new regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within the Office of Management on February 2, 2017, the administration indicated that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Our relationships with healthcare providers, physicians and third-party payers will be subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payers play a primary role in the recommendation and prescription of any product candidates that we develop for which we obtain marketing approval. Our future arrangements with third-party payers and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our medicines for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order, or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting or causing to be presented, to the federal government, claims for payment or approval from Medicare, Medicaid or other government payers that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at \$11,181 to \$22,363 per false claim;
- the federal Health Insurance Portability and Accountability Act of 1996, as further amended by the Health Information Technology for Economic and Clinical Health Act, which imposes certain requirements, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, healthcare clearinghouses and healthcare providers;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements under the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services, or HHS, information related to payments and other transfers of value to physicians and teaching hospitals and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payers, including private insurers, and certain state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures.

[Table of Contents](#)

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations and prospects.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Recently enacted and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of any product candidates we may develop, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payers.

The PPACA, which became law in 2010, contains provisions of importance to our business, including, without limitation, our ability to commercialize and the prices we may obtain for any product candidates we may develop and that are approved for sale, the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;

[Table of Contents](#)

- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012 became law, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the PPACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the PPACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the PPACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." The Congress may consider other legislation to replace elements of the PPACA during the next Congressional session.

Further, each chamber of the Congress has put forth multiple bills designed to repeal or repeal and replace portions of the PPACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the PPACA. The Congress will likely consider other legislation to replace elements of the PPACA, during the next Congressional session. It is possible that repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to repeal and replace PPACA provisions is highly uncertain in many respects, it is also possible that some of the PPACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with PPACA coverage expansion provision.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our potential products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payers.

The current presidential administration has also taken executive actions to undermine or delay implementation of the PPACA. Since January 2017, the president has signed two executive orders designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. One executive order directs federal agencies with authorities and responsibilities under the PPACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the PPACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers or manufacturers of pharmaceuticals or medical devices. The second executive order terminates the cost-sharing subsidies that reimburse insurers under the PPACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the PPACA for plans sold through such marketplaces. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than \$12 billion in PPACA risk corridor payments to third-party payers who argued were owed to them. On April 27, 2020, that decision was reversed by the U.S. Supreme Court.

The costs of prescription pharmaceuticals have also been the subject of considerable discussion in the United States, and members of Congress and the executive branch have stated that they will address such costs through new legislative and administrative measures. More recently, President Trump has issued several executive orders that are intended to lower the costs of prescription drug products. These orders direct the federal government to implement the initiatives outlined in the orders, meaning they will not have immediate effects.

To date, there have been several recent U.S. congressional inquiries and proposed federal and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. For example, the Trump Administration's 2021 budget proposal includes a \$135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients and increase patient access to lower cost generic drugs and biosimilars. In addition, on December 23, 2019, the Trump Administration published a proposed rulemaking that, if finalized, would allow states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants would be required to demonstrate their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. At the same time, the FDA issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for any product candidates we may develop or additional pricing pressures.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, consultants and partners, and, if we commence clinical trials, our principal investigators. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately, or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.

We are subject to numerous laws and regulations in each jurisdiction outside the United States in which we operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing

any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The Securities and Exchange Commission, or SEC, is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA and other anti-corruption laws potentially applicable to our business is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, compliance with the FCPA and other anti-corruption laws presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain drugs and drug candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the European Union General Data Protection Regulation (EU) 2016/679, or the GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States and, as a result, increases the scrutiny that such rules should apply to transfers of personal data from clinical trial sites located in the EEA to the United States. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and/or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater, and confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR provides that European Union member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with the GDPR's requirements are rigorous and time intensive and require significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

Similar privacy and data security requirements are either in place or underway in the United States. There are a broad variety of data protection laws that may be applicable to our activities, and a range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act of 2018, or the CCPA, which became effective on January 1, 2020, requires companies that process information on California residents to make new disclosures to consumers about their data collection, use and sharing practices, allow consumers to opt out of certain data sharing with third parties and provide a new cause of action for data breaches. Many other states are considering similar legislation, and a broad range of legislative measures also have been introduced at the federal level.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information, including certain laws that regulate the use and disclosure of personal health information. In particular, regulations promulgated pursuant to the Health Insurance Portability and Accountability Act of 1996, or HIPAA, establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. These provisions may be applicable to our business in the future. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation.

If we are unable to properly protect the privacy and security of protected health information, we could be found to have violated these privacy and security laws and/or breached certain contracts with our business partners. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems.

It is possible that new and existing laws may be interpreted and applied in a manner that is inconsistent with our practices and our efforts to comply with the evolving data protection rules may be unsuccessful. If so, this could result in government-imposed fines or penalties or orders requiring that we change our practices, which could adversely affect our business. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with federal, state and international laws regarding privacy and security of personal information could expose us to fines and penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. We also face a threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business, financial condition, results of operations or prospects.

Risks related to employee matters and managing growth

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the research and development, clinical, financial, operational and other business expertise of our executive officers, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment offer letters with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and sales and marketing personnel will also be critical to our success.

The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. Our success as a public company also depends on implementing and maintaining internal controls and the accuracy and timeliness of our financial reporting. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical, regulatory affairs and, if any product candidate we may develop receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

As a growing biotechnology company, we are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for and fully understanding the regulatory and manufacturing pathways to all of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize our product candidates, if approved, will depend in part on our ability to effectively manage the future development and expansion of our company.

Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure our stockholders that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with strategic partners as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;
- the need to implement or improve controls, procedures and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;

[Table of Contents](#)

- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or results of operations.

Our internal information technology systems, or those of our third-party vendors, collaborators or other contractors or consultants, may fail or suffer security breaches, loss or leakage of data and other disruptions, which could result in a material disruption of our product development programs, compromise sensitive information related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business.

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including but not limited to intellectual property, proprietary business information and personal information). It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information.

Despite the implementation of security measures, given the size and complexity of our internal information technology systems and those of our current and any future third-party vendors, collaborators and other contractors and consultants, and the increasing amounts of confidential information that they maintain, such information technology systems are vulnerable to damage or interruption from computer viruses, computer hackers, malicious code, employee theft or misuse, denial-of-service attacks, sophisticated nation-state and nation-state-supported actors, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We may not be able to anticipate all types of security threats, and we may not be able to implement preventive measures effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies.

While we seek to protect our information technology systems from system failure, accident and security breach, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary or confidential information or other disruptions. For example, the loss of clinical trial data from future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we were to experience a significant cybersecurity breach of our information systems or data, the costs associated with the investigation, remediation and potential notification of the breach to counterparties and data subjects could be material. In addition, our remediation efforts may not be successful. Moreover, if the information technology systems of our third-party vendors, collaborators and other contractors and consultants become subject to disruptions or security breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring. If we do not allocate and effectively manage the resources necessary to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, data loss or the loss of or damage to intellectual property or other proprietary information.

To the extent that any disruption or security breach were to result in a loss of, or damage to, our or our third-party vendors', collaborators' or other contractors' or consultants' data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability including litigation exposure, penalties and fines, we could become the subject of regulatory action or investigation based primarily on the laws and regulations discussed above in the privacy discussion, our competitive position and reputation could be harmed and the further development and commercialization of our product candidates could be delayed. Furthermore, any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our customers or employees, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects.

Our operations or those of the third parties upon whom we depend might be affected by the occurrence of a natural disaster, pandemic or other catastrophic event.

We depend on our employees, consultants, CDMOs, CLROs, as well as regulatory agencies and other parties, for the continued operation of our business. While we maintain disaster recovery plans, they might not adequately protect us. Despite any precautions we take for natural disasters or other catastrophic events, these events, including terrorist attack, pandemics, hurricanes, fire, floods and ice and snowstorms, could result in significant disruptions to our research and development, preclinical studies, clinical trials, and, ultimately, commercialization of our products. Long-term disruptions in the infrastructure caused by events, such as natural disasters, the outbreak of war, the escalation of hostilities and acts of terrorism or other “acts of God,” particularly involving cities in which we have offices, manufacturing or clinical trial sites, could adversely affect our businesses. Although we carry business interruption insurance policies and typically have provisions in our contracts that protect us in certain events, our coverage might not respond or be adequate to compensate us for all losses that may occur. Any natural disaster or catastrophic event affecting us, our CDMOs, our CLROs, regulatory agencies or other parties with which we are engaged could have a significant negative impact on our operations and financial performance.

Risks related to ownership of our common stock and our status as a public company

We do not know whether a market will continue to develop or be sustained for our common stock and, as a result, it may be difficult for our stockholders to sell shares of our common stock.

Although our common stock is listed on the Nasdaq Global Select Market, an active trading market for our shares may not continue to develop or be sustained. As a result, it may be difficult for our stockholders to sell their shares without depressing the market price for the shares or at all.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for our stockholders.

Our stock price is likely to be volatile. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price they paid for their shares. The market price for our common stock may be influenced by many factors, including:

- results of or developments in preclinical studies and clinical trials of any product candidates we may develop or those of our competitors or potential collaborators;
- timing of the results of our preclinical studies and clinical trials or those of our competitors;
- our success in commercializing any product candidates we may develop, if and when approved;
- the success of competitive products or technologies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other intellectual property or proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any product candidates we may develop;
- the results of our efforts to discover, develop, acquire or in-license products, product candidates, technologies or data referencing rights, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or the financial results of companies that are perceived to be similar to us;
- sales of common stock by us, our executive officers, directors or principal stockholders or others;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry, political and market conditions; and
- the other factors described in this “Risk Factors” section.

[Table of Contents](#)

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation has often been instituted against that company. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Such litigation may also cause us to incur other substantial costs to defend such claims and divert management's attention and resources.

If securities analysts do not publish or cease publishing research or reports or publish misleading, inaccurate or unfavorable research about our business or if they publish negative evaluations of our stock, the price and trading volume of our stock could decline.

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable research about our business, or provide more favorable relative recommendations about our competitors, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price and trading volume to decline.

The COVID-19 pandemic, which began in late 2019 and has spread worldwide, may affect our ability to initiate and complete preclinical studies, delay the initiation of our planned clinical trial or future clinical trials, disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, this pandemic has caused substantial disruption in the financial markets and may adversely impact economies worldwide, each of which could result in adverse effects on our business, on raising capital and on our operations.

The COVID-19 pandemic, which began in December 2019 and has spread worldwide, is causing many governments to implement measures to slow the spread of the outbreak through quarantines, travel restrictions, heightened border scrutiny and other measures. The outbreak and government measures taken in response have also had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. The future progression of the outbreak and its effects on our business and operations are uncertain. We and our CDMOs, and CROs, experienced a temporary reduction in the capacity to undertake research-scale production and to execute some preclinical studies, and may face disruptions in the future that affect our ability to initiate and complete preclinical studies, and disruptions in procuring items that are essential for our research and development activities, such as raw materials used in the manufacture of any product candidates we may develop, laboratory supplies used in our preclinical studies, or animals that are used for preclinical testing for which there are shortages because of ongoing efforts to address the outbreak. We and our CROs and CDMOs may also face disruptions related to our future IND-enabling studies and clinical trials arising from delays in preclinical studies, manufacturing disruptions, and the ability to obtain necessary IRB, IBC or other necessary site approvals, as well as other delays at clinical trial sites. The response to the COVID-19 pandemic may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions. The pandemic has already caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds through public offerings and may also impact the volatility of our stock price and trading in our stock. Moreover, it is possible the pandemic will significantly impact economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to adversely affect our business, financial condition, results of operations and prospects.

Unfavorable global economic conditions could adversely affect our business, financial condition, stock price and results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the 2008 global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the 2008 global financial crisis, could result in a variety of risks to our business, including, weakened demand for any product candidates we may develop and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive such difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business. Furthermore, our stock price may decline due in part to the volatility of the stock market and any general economic downturn.

Our executive officers, directors and principal stockholders, if they choose to act together, will continue to have the ability to control all matters submitted to stockholders for approval.

Our executive officers, directors and principal stockholders, in the aggregate, own shares representing more than 60% of our common stock as of October 31, 2020. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

This concentration of ownership control may:

- delay, defer or prevent a change in control;
- entrench our management and board of directors; or
- delay or prevent a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

We have broad discretion in the use of cash, cash equivalents and marketable securities and may not use them effectively.

Our management has broad discretion in the application of cash, cash equivalents and marketable securities and could spend them in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest these funds in a manner that does not produce income or that loses value.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be the sole source of gain for our stockholders.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of October 31, 2020, we have 46,516,475 shares of common stock outstanding, which includes the 12,105,263 shares of common stock that we sold in our IPO. The remaining 34,411,212 shares are currently restricted as a result of securities laws or lock-up agreements but will become eligible to be sold at various times after the expiration of the applicable lock-up period on December 9, 2020. The representatives of the underwriters may release some or all of the shares of common stock subject to lock-up agreements at any time and without notice, which would allow for earlier sales of shares in the public market.

Moreover, beginning on December 9, 2020, holders of an aggregate of 31,887,648 shares of our common stock will have rights subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We have also filed a registration statement on Form S-8 to register shares of common stock that we may issue under our equity compensation plans. Shares registered under this registration statement on Form S-8 can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates, vesting arrangements and exercise of options, and the lock-up agreements entered into in connection with our IPO.

We are an “emerging growth company,” and a “smaller reporting company” and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an “emerging growth company,” or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We may remain an EGC until December 31, 2025, although if the market value of our common stock that is held by non-affiliates exceeds \$700.0 million as of any June 30 before that time or if we have annual gross revenues of \$1.07 billion or more in any fiscal year, we would cease to be an EGC as of December 31 of the applicable year. We also would cease to be an EGC if we issue more than \$1.0 billion of non-convertible debt over a three-year period. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

[Table of Contents](#)

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

Even after we no longer qualify as an emerging growth company, we may continue to qualify as a "smaller reporting company," which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. In addition, if we are a smaller reporting company with less than \$100 million in annual revenue, we would not be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404.

We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to take advantage of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either irrevocably elect to "opt out" of such extended transition period or no longer qualify as an EGC. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

We have incurred and will continue to incur increased costs as a result of operating as a newly public company, and our management has devoted and will continue to be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an EGC or a smaller reporting company, we will incur significant legal, accounting and other expenses that we did not previously incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote and will continue to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs, particularly as we hire additional financial and accounting employees to meet public company internal control and financial reporting requirements and will make some activities more time-consuming and costly compared to when we were a private company.

We cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting beginning with our filing of an Annual Report on Form 10-K with the SEC for the year ended December 31, 2021. However, while we remain an EGC or a smaller reporting company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we are engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, including through hiring additional financial and accounting personnel, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal control over financial reporting is necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, is designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could harm our business and have a negative effect on the trading price of our stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management is required to assess the effectiveness of these controls annually. However, for as long as we are an EGC under the JOBS Act or a smaller reporting company, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404. We could be an EGC for up to five years. An independent assessment of the effectiveness of our internal control over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal control over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation, which could have a negative effect on the trading price of our stock.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to certain reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition.

Recent changes in tax law may adversely affect our business or financial condition. On December 22, 2017, the U.S. government enacted the Tax Act, which significantly reformed the Code. The Tax Act, among other things, contained significant changes to corporate taxation, including a reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, the limitation of the tax deduction for net interest expense to 30% of adjusted taxable income (except for certain small businesses), the limitation of the deduction for NOLs arising in taxable years beginning after December 31, 2017 to 80% of current year taxable income and elimination of NOL carrybacks for losses arising in taxable years ending after December 31, 2017 (though any such NOLs may be carried forward indefinitely), the imposition of a one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, the elimination of U.S. tax on foreign earnings (subject to certain important exceptions), the allowance of immediate deductions for certain new investments instead of deductions for depreciation expense over time, and the modification or repeal of many business deductions and credits.

As part of Congress' response to the COVID-19 pandemic, the Families First Coronavirus Response Act, or FFCR Act, was enacted on March 18, 2020, and the CARES Act was enacted on March 27, 2020. Both contain numerous tax provisions. In particular, the CARES Act retroactively and temporarily (for taxable years beginning before January 1, 2021) suspends application of the 80%-of-income limitation on the use of NOLs, which was enacted as part of the Tax Act. It also provides that NOLs arising in any taxable year beginning after December 31, 2017, and before January 1, 2021 are generally eligible to be carried back up to five years. The CARES Act also temporarily (for taxable years beginning in 2019 or 2020) relaxes the limitation of the tax deductibility for net interest expense by increasing the limitation from 30 to 50% of adjusted taxable income.

Regulatory guidance under the Tax Act, the FFCR Act and the CARES Act is and continues to be forthcoming, and such guidance could ultimately increase or lessen impact of these laws on our business and financial condition. It is also likely that Congress will enact additional legislation in connection with the COVID-19 pandemic, some of which could have an impact on our company. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the FFCR Act or the CARES Act.

[Table of Contents](#)

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current directors and members of management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our certificate of incorporation designates the state courts in the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against the company and our directors, officers and employees.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for the following types of proceedings:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders;
- any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; or
- any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine.

These choice of forum provisions will not apply to suits brought to enforce a duty or liability created by the Securities Act of 1933, as amended, the Exchange Act or any other claim for which federal courts have exclusive jurisdiction. Furthermore, our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States of America shall, to the fullest extent permitted by law, be the sole and exclusive forum for the resolution of any claims arising under the Securities Act.

These exclusive forum provisions may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our

[Table of Contents](#)

directors, officers and employees. Alternatively, if a court were to find the choice of forum provisions contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could materially adversely affect our business, financial condition and operating results.

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

Use of Proceeds from Initial Public Offering

On June 16, 2020, we closed our initial public offering, or IPO, of our common stock pursuant to which we issued and sold 12,105,263 shares of our common stock, including 1,578,947 shares sold by us pursuant to the full exercise of the underwriters' option to purchase additional shares at a price to the public of \$19.00 per share for aggregate gross proceeds of \$230.0 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. 333-238608), which was declared effective by the SEC on June 11, 2020. J.P. Morgan Securities LLC, Jefferies LLC and Cowen and Company, LLC acted as joint book-running managers and Wedbush PacGrow acted as lead manager of our IPO.

We received aggregate net proceeds of approximately \$210.7 million after deducting underwriting discounts and commissions and other offering expenses payable by us. None of the underwriting discounts and commissions or offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any of our affiliates.

As of September 30, 2020, we have used approximately \$20.0 million of net offering proceeds. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on June 12, 2020.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) of the Securities Exchange Act, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) of the Securities Exchange Act, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1**	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2**	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
*	Filed herewith.
**	Furnished herewith.
†	Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.
+	Indicates management contract or compensatory plan.

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.

GENERATION BIO CO.

Date: November 10, 2020

By: /s/ Geoff McDonough

Geoff McDonough, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 10, 2020

By: /s/ Matthew Norkunas

Matthew Norkunas, M.D., M.B.A.
Chief Financial Officer
(Principal Financial and Accounting Officer)

**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Geoff McDonough, hereby certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Generation Bio Co.;

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 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)) 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) 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

(c) 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 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: November 10, 2020

/s/ Geoff McDonough

Geoff McDonough, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

**CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Matthew Norkunas, hereby certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Generation Bio Co.;

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 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)) 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) 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

(c) 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 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: November 10, 2020

/s/ Matthew Norkunas

Matthew Norkunas, M.D., M.B.A.

Chief Financial Officer

(Principal Financial and Accounting Officer)

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

I, Geoff McDonough, certify pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge, the Quarterly Report on Form 10-Q of Generation Bio Co. for the fiscal quarter ended September 30, 2020 fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Generation Bio Co.

/s/ Geoff McDonough

Geoff McDonough, M.D.

President and Chief Executive Officer

(Principal Executive Officer)

November 10, 2020

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

I, Matthew Norkunas, certify pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge, the Quarterly Report on Form 10-Q of Generation Bio Co. for the fiscal quarter ended September 30, 2020 fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Generation Bio Co.

/s/ Matthew Norkunas

Matthew Norkunas, M.D., M.B.A.

Chief Financial Officer

(Principal Financial and Accounting Officer)

November 10, 2020