
**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549**

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2018

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: 001-38707

LogicBio Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware
(State or other jurisdiction of
incorporation or organization)

47-1514975
(I.R.S. Employer
Identification No.)

**610 Main Street, 3rd Floor
Cambridge, MA 02139
(617) 245-0399**

(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
Emerging growth company	<input checked="" type="checkbox"/>		

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

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

As of November 30, 2018, the registrant had 22,176,129 shares of common stock, \$0.0001 par value per share, outstanding.

Table of Contents

	<u>Page</u>
PART I. FINANCIAL INFORMATION	
Item 1. Condensed Consolidated Financial Statements (Unaudited)	4
Condensed Consolidated Balance Sheets as of September 30, 2018 and December 31, 2017 (Unaudited)	4
Condensed Consolidated Statements of Operations for the Three and Nine Months Ended September 30, 2018 and 2017 (Unaudited)	5
Condensed Consolidated Statements of Comprehensive Loss for the Three and Nine months Ended September 30, 2018 and 2017 (Unaudited)	6
Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Deficit for the Three and Nine Months Ended September 30, 2018 and 2017 (Unaudited)	7
Condensed Consolidated Statements of Cash Flows for the Nine Months Ended September 30, 2018 and 2017 (Unaudited)	8
Notes to Condensed Consolidated Financial Statements (Unaudited)	9
Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations	16
Item 3. Quantitative and Qualitative Disclosures About Market Risk	25
Item 4. Controls and Procedures	26
PART II. OTHER INFORMATION	
Item 1. Legal Proceedings	27
Item 1A. Risk Factors	27
Item 2. Unregistered Sales of Equity Securities and Use of Proceeds	84
Item 6. Exhibits	84
Signatures	85

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements. In some cases, you can identify these statements by forward-looking words such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “could,” “would,” “project,” “plan,” “expect,” or similar expressions, or the negative or plural of these words or expressions. These forward-looking statements include statements concerning the following:

- the initiation, cost, timing, progress and results of our current and future research and development activities and preclinical studies and potential future clinical trials;
- potential attributes and benefits of our GeneRide technology platform and our product candidates;
- our ability to take advantage of the modular nature of our GeneRide platform to simplify and accelerate development of new product candidates;
- the potential benefits of strategic partnership agreements and our ability to enter into selective strategic partnership arrangements;
- the timing of, and our ability to obtain and maintain, regulatory approvals for our product candidates;
- our ability to quickly and efficiently identify and develop additional product candidates;
- our ability to advance any product candidate into and successfully complete clinical trials;
- our intellectual property position, including with respect to our trade secrets and the duration of our patent protection; and
- our estimates regarding expenses, future revenues, capital requirements, the sufficiency of our current and expected cash resources and our need for additional financing.

These statements are only current predictions and are subject to known and unknown risks, uncertainties and other factors that may cause our or our industry’s actual results, levels of activity, performance or achievements to be materially different from those anticipated by the forward-looking statements. We discuss many of these risks in greater detail under the heading “Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q. You should not rely upon forward-looking statements as predictions of future events.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. Except as required by law, we are under no duty to update or revise any of the forward-looking statements in this Quarterly Report on Form 10-Q, whether as a result of new information, future events or otherwise, after the date of this Quarterly Report on Form 10-Q.

Unless the context otherwise requires, the terms “LogicBio,” “LogicBio Therapeutics Inc.,” the “Company,” “we,” “us,” “our” and similar references in this Quarterly Report on Form 10-Q refer to LogicBio Therapeutics Inc. and its subsidiaries.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

LogicBio Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(In thousands, except share and per share data)
(Unaudited)

	<u>September 30,</u> <u>2018</u>	<u>December 31,</u> <u>2017</u>
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	\$ 12,877	\$ 24,575
Prepaid expenses and other current assets	1,726	1,118
Total current assets	14,603	25,693
Property and equipment, net	487	232
Other assets	1,906	249
TOTAL ASSETS	<u>\$ 16,996</u>	<u>\$ 26,174</u>
LIABILITIES, CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT		
CURRENT LIABILITIES:		
Accounts payable	\$ 983	\$ 1,112
Accrued expenses and other current liabilities	1,566	599
Total current liabilities	2,549	1,711
Total liabilities	2,549	1,711
COMMITMENTS AND CONTINGENCIES		
Series A convertible preferred stock, par value of \$0.0001 per share; 3,645,848 shares authorized as of September 30, 2018 and December 31, 2017; 2,976,190 shares issued and outstanding as of September 30, 2018 and December 31, 2017; liquidation and redemption value of \$5,403 and \$5,137 as of September 30, 2018 and December 31, 2017, respectively	4,359	4,359
Series B convertible preferred stock, par value of \$0.0001 per share; 30,063,791 shares authorized as of September 30, 2018 and December 31, 2017; 19,541,465 shares issued and outstanding as of September 30, 2018 and December 31, 2017; liquidation and redemption value of \$32,175 and \$30,428 as of September 30, 2018 and December 31, 2017, respectively	28,703	28,703
STOCKHOLDERS' DEFICIT:		
Common stock, par value of \$0.0001 per share; 47,493,828 and 45,493,828 shares authorized as of September 30, 2018 and December 31, 2017, respectively; 2,176,075 and 1,606,358 shares issued and outstanding as of September 30, 2018 and December 31, 2017, respectively	1	1
Additional paid-in capital	1,566	1,035
Accumulated other comprehensive loss	(1)	(14)
Accumulated deficit	(20,181)	(9,621)
Total stockholders' deficit	(18,615)	(8,599)
TOTAL LIABILITIES, CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT	<u>\$ 16,996</u>	<u>\$ 26,174</u>

See notes to consolidated financial statements.

LogicBio Therapeutics, Inc.
Condensed Consolidated Statements of Operations
(In thousands, except share and per share data)
(Unaudited)

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2018</u>	<u>2017</u>	<u>2018</u>	<u>2017</u>
OPERATING EXPENSES:				
Research and development	\$ 2,432	\$ 580	\$ 6,113	\$ 1,467
General and administrative	2,119	470	4,453	1,303
Total operating expenses	<u>4,551</u>	<u>1,050</u>	<u>10,566</u>	<u>2,770</u>
LOSS FROM OPERATIONS	<u>(4,551)</u>	<u>(1,050)</u>	<u>(10,566)</u>	<u>(2,770)</u>
OTHER (EXPENSE) INCOME, NET:				
Interest income (expense), net	74	(2)	202	(13)
Other (expense) income, net	(154)	37	(158)	49
Total other (expense) income, net	<u>(80)</u>	<u>35</u>	<u>44</u>	<u>36</u>
Loss before income taxes	(4,631)	(1,015)	(10,522)	(2,734)
Income tax provision	(38)	(29)	(38)	(48)
Net loss	<u>\$ (4,669)</u>	<u>\$ (1,044)</u>	<u>\$ (10,560)</u>	<u>\$ (2,782)</u>
Net loss attributable to common stockholders—basic and diluted (Note 9)	<u>\$ (8,621)</u>	<u>\$ (2,306)</u>	<u>\$ (14,512)</u>	<u>\$ (4,044)</u>
Net loss per share attributable to common stockholders—basic and diluted	<u>\$ (4.03)</u>	<u>\$ (1.55)</u>	<u>\$ (7.39)</u>	<u>\$ (3.07)</u>
Weighted-average common stock outstanding—basic and diluted	<u>2,138,160</u>	<u>1,483,268</u>	<u>1,963,976</u>	<u>1,319,192</u>

See notes to consolidated financial statements.

LogicBio Therapeutics, Inc.
Condensed Consolidated Statements of Comprehensive Loss
(In thousands)
(Unaudited)

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2018</u>	<u>2017</u>	<u>2018</u>	<u>2017</u>
Net loss	\$ (4,669)	\$ (1,044)	\$ (10,560)	\$ (2,782)
Other comprehensive income (loss):				
Foreign currency translation adjustment	\$ 5	\$ 33	\$ 13	\$ (9)
Comprehensive loss	<u>\$ (4,664)</u>	<u>\$ (1,011)</u>	<u>\$ (10,547)</u>	<u>\$ (2,791)</u>

See notes to consolidated financial statements.

LogicBio Therapeutics, Inc.
Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Deficit
(In thousands, except share data)
(Unaudited)

	Convertible Preferred Stock				Common Stock \$0.0001 Par Value		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Deficit
	\$0.0001 Par Value Series A		\$0.0001 Par Value Series B		Shares	Amount				
	Shares	Amount	Shares	Amount	Shares	Amount				
BALANCE, January 1, 2017	2,976,190	\$ 4,359	—	\$ —	978,881	\$ 1	\$ 628	\$ —	\$ (3,879)	\$ (3,250)
Issuance of Series B convertible preferred stock, net of issuance costs of \$478	—	—	19,541,465	28,703	—	—	—	—	—	—
Vesting of restricted stock	—	—	—	—	333,563	—	—	—	—	—
Foreign currency translation adjustment	—	—	—	—	—	—	—	(42)	—	(42)
Stock-based compensation expense	—	—	—	—	—	—	101	—	—	101
Adoption of ASU 2018-07	—	—	—	—	—	—	(53)	—	53	—
Net loss	—	—	—	—	—	—	—	—	(1,738)	(1,738)
BALANCE, June 30, 2017	2,976,190	\$ 4,359	19,541,465	\$ 28,703	1,312,444	\$ 1	\$ 676	\$ (42)	\$ (5,564)	\$ (4,929)
Vesting of restricted stock	—	—	—	—	146,958	—	—	—	—	—
Foreign currency translation adjustment	—	—	—	—	—	—	—	33	—	33
Stock-based compensation expense	—	—	—	—	—	—	48	—	—	48
Net loss	—	—	—	—	—	—	—	—	(1,044)	(1,044)
BALANCE, September 30, 2017	2,976,190	\$ 4,359	19,541,465	\$ 28,703	1,459,402	\$ 1	\$ 724	\$ (9)	\$ (6,608)	\$ (5,892)
BALANCE, December 31, 2017	2,976,190	\$ 4,359	19,541,465	\$ 28,703	1,606,360	\$ 1	\$ 1,035	\$ (14)	\$ (9,621)	\$ (8,599)
Vesting of restricted stock	—	—	—	—	336,286	—	—	—	—	—
Foreign currency translation adjustment	—	—	—	—	—	—	—	8	—	8
Stock-based compensation expense	—	—	—	—	—	—	233	—	—	233
Net loss	—	—	—	—	—	—	—	—	(5,891)	(5,891)
BALANCE, June 30, 2018	2,976,190	\$ 4,359	19,541,465	\$ 28,703	1,942,646	\$ 1	\$ 1,268	\$ (6)	\$ (15,512)	\$ (14,249)
Vesting of restricted stock	—	—	—	—	160,329	—	—	—	—	—
Exercise of options	—	—	—	—	17,003	—	11	—	—	11
Issuance of common stock	—	—	—	—	56,097	—	—	—	—	—
Foreign currency translation adjustment	—	—	—	—	—	—	—	5	—	5
Stock-based compensation expense	—	—	—	—	—	—	287	—	—	287
Net loss	—	—	—	—	—	—	—	—	(4,669)	(4,669)
BALANCE, September 30, 2018	2,976,190	\$ 4,359	19,541,465	\$ 28,703	2,176,075	\$ 1	\$ 1,566	\$ (1)	\$ (20,181)	\$ (18,615)

See notes to consolidated financial statements.

LogicBio Therapeutics, Inc.
Condensed Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	<u>Nine Months Ended September 30,</u>	
	<u>2018</u>	<u>2017</u>
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	\$ (10,560)	\$ (2,782)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	49	30
Loss on disposal of property and equipment	142	—
Stock-based compensation expense	520	149
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(608)	(258)
Other non-current assets	225	(10)
Accounts payable	(570)	129
Accrued expenses and other current liabilities	175	(215)
Net cash used in operating activities	<u>(10,627)</u>	<u>(2,957)</u>
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of property and equipment	(362)	(36)
Disposal of property and equipment	35	—
Net cash used in investing activities	<u>(327)</u>	<u>(36)</u>
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of Series B preferred stock	—	29,181
Payment of issuance costs	—	(478)
Proceeds from exercise of stock options	11	—
Payment of deferred initial public offering costs	(775)	—
Net cash (used in) provided by financing activities	<u>(764)</u>	<u>28,703</u>
Effect on foreign exchange rates on cash and cash equivalents	20	(26)
NET (DECREASE) INCREASE IN CASH AND CASH EQUIVALENTS	(11,698)	25,684
Cash and cash equivalents at beginning of year	24,575	1,728
Cash and cash equivalents at end of year	<u>\$ 12,877</u>	<u>\$ 27,412</u>
SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION:		
Cash paid for interest	\$ —	\$ 13
Cash paid for taxes	100	4
Property and equipment purchases in accounts payable and accrued expenses	126	30
Deferred initial offering costs in accounts payable and accrued expenses	1,107	—

See notes to consolidated financial statements.

LogicBio Therapeutics, Inc.
Notes to Condensed Consolidated Financial Statements
(In thousands, except share and per share data)
(Unaudited).

1. NATURE OF BUSINESS AND BASIS OF PRESENTATION

LogicBio Therapeutics, Inc. (“LogicBio” or the “Company”) was incorporated in 2014 as a Delaware corporation. Its principal offices are in Cambridge, Massachusetts. The Company is a genome editing company focused on developing medicines to durably treat rare diseases in patients with significant unmet medical need, using GeneRide, its proprietary technology platform. GeneRide technology is designed to precisely and stably integrate corrective genes into a patient’s genome to provide a durable therapeutic effect. The Company has demonstrated proof of concept of its therapeutic platform in animal models for a number of diseases and is focusing on its lead product candidate, LB-001, for the treatment of Methylmalonic Acidemia, a life-threatening disease that presents at birth.

Since its inception, the Company has devoted the majority of its efforts to business planning, research and development, developing markets, raising capital, recruiting management and technical staff. The Company is subject to a number of risks similar to those of other companies conducting high-risk, early-stage research and development of product candidates. Principal among these risks are a dependency on key individuals and intellectual property, competition from other products and companies, and the technical risks associated with the successful research, development and clinical manufacturing of its product candidates. The Company’s success is dependent upon its ability to continue to raise additional capital in order to fund ongoing research and development, meet its obligations and, ultimately, obtain regulatory approval of its products, successfully commercialize its products, generate revenue and, attain profitable operations.

Through September 30, 2018, the Company had funded its operations primarily with proceeds from the sale of convertible preferred stock (see Note 6). On October 23, 2018, the Company completed an initial public offering (“IPO”) in which the Company issued and sold 8,050,000 shares of its common stock, including 1,050,000 shares pursuant to the full exercise of the underwriters’ option to purchase additional shares, at a public offering price of \$10.00 per share, for aggregate gross proceeds of \$80,500. The Company received approximately \$72,400 in net proceeds after deducting underwriting discounts and commissions and offering costs.

In connection with the IPO, the Company effected a one-for-1.90993 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for the Company’s convertible preferred stock. The reverse stock split became effective on October 8, 2018. Accordingly, all share and per share amounts of common stock for all periods presented in the accompanying condensed consolidated financial statements and notes thereto have been retroactively adjusted, where applicable, to reflect this reverse stock split and adjustment of the preferred stock conversion ratios.

Upon the closing of the IPO, all of the outstanding shares of convertible preferred stock automatically converted into 11,789,775 shares of common stock at the applicable conversion ratio then in effect. Subsequent to the closing of the IPO, there were no shares of convertible preferred stock outstanding. The financial statements as of September 30, 2018, including share and per share amounts, do not give effect to the IPO, as it closed subsequent to September 30, 2018.

[Table of Contents](#)

Management believes that the Company's existing cash and cash equivalents, together with the net proceeds from the IPO, will allow the Company to continue its operations through 2020. In the absence of a significant source of recurring revenue, the continued viability of the Company beyond that point is dependent on its ability to continue to raise additional capital to finance its operations. There can be no assurance that the Company will be able to obtain sufficient capital to cover its costs on acceptable terms, if at all.

The accompanying unaudited condensed consolidated financial statements as of September 30, 2018 and for the three and nine months ended September 30, 2018 and 2017 have been prepared by the Company in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP") and, 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 U.S. GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These condensed consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto for the year ended December 31, 2017 included in the Company's final prospectus that forms a part of the Company's Registration Statement on Form S-1 (Reg. No. 333-227523), filed with the SEC pursuant to Rule 424(b)(4) on October 22, 2018 (the "Prospectus").

The unaudited interim condensed consolidated financial statements have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the accompanying unaudited interim condensed consolidated financial statements contain all adjustments which are necessary for a fair statement of the Company's financial position as of September 30, 2018, consolidated results of operations for the three and nine months ended September 30, 2018 and 2017 and cash flows for the nine months ended September 30, 2018 and 2017. Such adjustments are of a normal and recurring nature. The results of operations for the three and nine months ended September 30, 2018 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2018.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Significant Accounting Policies

The Company's significant accounting policies are disclosed in the audited consolidated financial statements for the year ended December 31, 2017 and the notes thereto, which are included in the Company's Prospectus. Since the date of those financial statements, there have been no material changes to its significant accounting policies.

Principles of Consolidation

In January 2016, the Company formed LogicBio Research, a wholly owned Israeli subsidiary, for the purpose of conducting research and development activities on the Company's behalf. As of September 30, 2018, all operations had ceased for LogicBio Research. In April 2018, the Company formed LogicBio Australia Pty Limited ("LogicBio Australia"), a wholly owned Australian subsidiary, for the purpose of conducting research and development activities on the Company's behalf. As of September 30, 2018, no activity had commenced for LogicBio Australia. The accompanying condensed consolidated financial statements include the accounts of the Company and LogicBio Research. All intercompany accounts and transactions have been eliminated in consolidation.

Deferred Offering Costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' (deficit) equity as a reduction of proceeds generated as a result of the offering.

[Table of Contents](#)

Should a planned equity financing be abandoned, the deferred offering costs would be expensed immediately as a charge to operating expenses in the condensed consolidated statement of operations. The Company recorded deferred offering costs, related to the IPO, of \$1,882 as of September 30, 2018 and are included in other assets. Upon closing the IPO in October 2018, deferred offering costs were derecognized and recorded against the IPO proceeds as a debit to additional paid-in capital.

Recently Adopted Accounting Pronouncements

In May 2014, the FASB issued Accounting Standards Update (“ASU”) No. 2014-09, “Revenue from Contracts with Customers (Topic 606) (ASU No. 2014-09),” which modifies how all entities recognize revenue, and consolidates into one ASC (ASC Topic 606, Revenue from Contracts with Customers) the current guidance found in ASC Topic 605, and various other revenue accounting standards for specialized transactions and industries. ASU No. 2014-09 outlines a comprehensive five-step revenue recognition model based on the principle that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. ASU No. 2014-09 may be applied using either a full retrospective approach, under which all years included in the financial statements will be presented under the revised guidance, or a modified retrospective approach, under which financial statements will be prepared under the revised guidance for the year of adoption, but not for prior years. Under the latter method, entities will recognize a cumulative catch-up adjustment to the opening balance of retained earnings at the effective date for contracts that still require performance by the entity at the date of adoption.

In August 2015, the FASB issued ASU No. 2015-14, “Revenue from Contracts with Customers (Topic 606): Deferral of Effective Date (ASU No. 2015-14),” which defers the effective date of ASU No. 2014-09 by one year. The guidance will be effective for the Company for annual reporting period beginning after December 15, 2018. To date, the Company has not had any arrangements that are within the scope of ASU No. 2014-09, or its predecessor, ASC Topic 605. Adoption of ASU No. 2014-09 did not have any impact on the Company’s financial statements.

In May 2017, the FASB issued ASU No. 2017-09, “Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting,” which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. The standard is effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted. The implementation of ASU 2017-09 did not have a material effect on the Company’s consolidated financial statements.

In June 2018, the FASB issued ASU No. 2018-07, “Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting.” These amendments expand the scope of Topic 718, Compensation—Stock Compensation (which currently only includes share-based payments to employees) to include share-based payments issued to nonemployees for goods or services. Consequently, the accounting for share-based payments to nonemployees and employees will be substantially aligned. The ASU supersedes Subtopic 505-50, Equity—Equity-Based Payments to Non-Employees. This standard is effective for public companies for annual periods beginning after December 15, 2018, including interim periods within those fiscal years, with early adoption permitted as long as ASU No. 2014-09 has been adopted by the Company. Adoption of ASU No. 2018-07 did not have a material impact on the Company’s financial statements.

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, “Leases (Topic 842),” which requires an entity to recognize assets and liabilities arising from a lease for both financing and operating leases. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. ASU No. 2016-02 is effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The Company is currently in the process of evaluating the impact of the adoption of ASU No. 2016-02 on the Company’s financial statements.

Table of Contents

In December 2016, the FASB issued ASU No. 2016-18, "Statement of Cash Flows (Topic 230): Restricted Cash." ASU No. 2016-18 provides guidance on the classification and presentation of changes in restricted cash and cash equivalents in the statement of cash flows. ASU No. 2016-18 is effective for fiscal years beginning in 2019 with early adoption permitted. The Company does not expect the adoption of ASU No. 2016-18 to have a significant impact on its statements of cash flows because the Company does not have restricted cash.

3. FAIR VALUE MEASUREMENTS

The following tables present information about the Company's financial assets and liabilities measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values:

Description	September 30, 2018	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Other Observable Inputs (Level 3)
<i>Assets</i>				
Sweep bank account	\$ 12,600	\$ 12,600	\$ —	\$ —
Total financial assets	\$ 12,600	\$ 12,600	\$ —	\$ —

Description	December 31, 2017	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Other Observable Inputs (Level 3)
<i>Assets</i>				
Sweep bank account	\$ 24,415	\$ 24,415	\$ —	\$ —
Total financial assets	\$ 24,415	\$ 24,415	\$ —	\$ —

There have been no transfers between fair value measure levels during the three and nine months ended September 30, 2018.

4. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other current liabilities at September 30, 2018 and December 31, 2017 consisted of the following:

	September 30, 2018	December 31, 2017
Accrued compensation and benefits	\$ 143	\$ 290
Accrued insurance	32	20
Accrued professional services	998	64
Other	393	225
Total accrued expenses and other current liabilities	\$ 1,566	\$ 599

Accrued compensation and benefits consists primarily of accrued bonuses, accrued commissions, and accrued vacation. Accrued professional services consists primarily of consulting services and legal services.

5. SIGNIFICANT AGREEMENTS

Research Agreement

In August 2018, the Company entered into a new research agreement with a contract research organization to pay for services related to certain research and development activities relevant to its programs to be identified in supplements to the research agreement. In September 2018, two supplements to the research agreement were entered into. The Company recorded \$258 as deferred research and development expenses related to this agreement as of September 30, 2018, included in prepaid expenses and other current assets.

6. CONVERTIBLE PREFERRED STOCK

Series A Preferred Stock and Series B convertible preferred stock (“Series B Preferred Stock”) is collectively referred to as “Preferred Stock.” As of September 30, 2018 and December 31, 2017, Preferred Stock consisted of the following:

	September 30, 2018				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Value	Common stock Issuable Upon Conversion
Series A Preferred Stock	3,645,848	2,976,190	\$ 4,359	\$ 5,403	1,558,271
Series B Preferred Stock	30,063,791	19,541,465	\$28,703	\$ 32,175	10,231,504
	<u>33,709,639</u>	<u>22,517,655</u>	<u>\$33,062</u>	<u>\$ 37,578</u>	<u>11,789,775</u>

	December 31, 2017				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Value	Common stock Issuable Upon Conversion
Series A Preferred Stock	3,645,848	2,976,190	\$ 4,359	\$ 5,137	1,558,271
Series B Preferred Stock	30,063,791	19,541,465	28,703	30,428	10,231,504
	<u>33,709,639</u>	<u>22,517,655</u>	<u>\$33,062</u>	<u>\$ 35,565</u>	<u>11,789,775</u>

On October 23, 2018, upon the closing of the Company’s IPO, all outstanding shares of Preferred Stock converted into 11,789,775 shares of the Company’s common stock.

The following is a summary of the rights and privileges of the preferred stockholders as of September 30, 2018.

Conversion: Each share of Preferred Stock was convertible, at the option of the holder, at any time, into shares of common stock on a one-for-1.90993 basis. The conversion ratio was determined by dividing the original issue price of \$1.4933 by the conversion price of \$0.78186. The Preferred Stock would automatically convert into shares of common stock at the closing a Qualified IPO (as defined in the Company’s Amended and Restated Certificate of incorporation, as amended from time to time) or in a non-Qualified IPO, upon the approval of at least 60% of the Preferred Stockholders.

Liquidation Preference: In the event of any liquidation or “Deemed Liquidation Event,” defined below, the preferred stockholders would have been entitled to the greater of (i) the original issue price of the Preferred Stock plus any accrued dividends not yet paid plus any other dividends declared and unpaid or (ii) the amount payable had all classes of shares been converted to common stock. In the event of a Deemed Liquidation Event, accrued dividends would not exceed 40% of the original issue price. After payments of all preferential amounts are made to the Preferred Stockholders, any remaining assets would be distributed to the common stockholders on a pro rata basis. A Deemed Liquidation Event was defined as a merger, consolidation, reorganization or similar transaction; the sale transfer, exclusive license of all or substantially all of the Company’s assets/intellectual property; or the sale or transfer of shares to any person (or group of related or affiliated persons), directly or indirectly, representing a greater than 50% of the voting power of the voting securities of the Company.

Dividends: Dividends accrued at a rate of 8.0% of the original issue price per year, per share, on the anniversary of the issuance date. Dividends were cumulative; however, accrued dividends would be payable only if and when declared by the board of directors. Dividends on other classes of the Company’s stock (other than dividends on shares of common stock payable in shares of common stock) were not to be declared or paid unless the Preferred Stockholders were first paid (i) all dividends accrued and not yet paid plus (ii) the product of (a) dividends declared on an as converted basis and (b) Preferred Stock on an as converted basis. No dividends had been declared through September 30, 2018.

Voting Rights: Preferred Stock and common stock voted together as one class on an as converted basis.

7. STOCK-BASED COMPENSATION

2014 Plan

On June 25, 2018, the Company increased the number of shares available for future grant under the 2014 Plan. At September 30, 2018, there were 977,522 shares available for future grant under the 2014 Plan.

Total stock-based compensation expense recorded as research and development and general and administrative expenses, respectively, for employees, directors and non-employees for the three and nine months ended September 30, 2018 and 2017 is as follows:

	Three Months Ended September		Nine Months Ended September	
	2018	2017	2018	2017
Research and development	\$ 51	\$ 41	\$ 147	\$ 119
General and administrative	236	7	373	30
Total stock-based compensation expense	<u>\$ 287</u>	<u>\$ 48</u>	<u>\$ 520</u>	<u>\$ 149</u>

During the nine months ended September 30, 2018, the Company granted options to purchase 596,394 shares of common stock. The Company recorded stock-based compensation expense for options granted of \$207 and \$292 during the three and nine months ended September 30, 2018, respectively. During the nine months ended September 30, 2018, the Company granted 107,054 shares of restricted stock. The Company recorded stock-based compensation expense for restricted stock granted of \$80 and \$228 during the three and nine months ended September 30, 2018, respectively.

As of September 30, 2018, there were 2,009,671 options outstanding. The weighted-average grant date fair value per share of options granted during the nine months ended September 30, 2018 was \$4.20. As of September 30, 2018, there was \$2,583 of unrecognized stock-based compensation expense related to unvested stock options. The unrecognized stock-based compensation expense is estimated to be recognized over a period of 2.4 years at September 30, 2018.

As of September 30, 2018, there were 1,045,050 shares of unvested restricted stock outstanding. The weighted-average grant date fair value per share of restricted stock granted during the nine months ended September 30, 2018 was \$4.02. As of September 30, 2018, there was \$604 of unrecognized stock-based compensation expense related to unvested restricted stock. The unrecognized stock-based compensation expense is estimated to be recognized over a period of 1.1 years at September 30, 2018.

8. INCOME TAXES

During the nine months ended September 30, 2018 and year ended December 31, 2017, the Company recorded a full valuation allowance on federal and state deferred tax assets since management does not forecast the Company to be in a profitable position in the near future.

[Table of Contents](#)**9. LOSS PER SHARE**

Basic loss per share is computed by dividing net loss attributable to common stockholders by the weighted-average common shares outstanding:

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2018</u>	<u>2017</u>	<u>2018</u>	<u>2017</u>
Numerator:				
Net loss	\$ (4,669)	\$ (1,044)	\$ (10,560)	\$ (2,782)
Less: Accruals of dividends of preferred stock	(3,952)	(1,262)	(3,952)	(1,262)
Net loss attributable to common stockholders - basic and diluted	<u>(8,621)</u>	<u>(2,306)</u>	<u>(14,512)</u>	<u>(4,044)</u>
Denominator:				
Weighted-average common stock outstanding	<u>2,138,160</u>	<u>1,483,268</u>	<u>1,963,976</u>	<u>1,319,192</u>
Net loss per share attributable to common stockholders - basic and diluted	<u>\$ (4.03)</u>	<u>\$ (1.55)</u>	<u>\$ (7.39)</u>	<u>\$ (3.07)</u>

The Company's potential dilutive securities, which included Preferred Stock, restricted stock, and stock options, were excluded from the computation of diluted net loss per share as the effect would be anti-dilutive. Therefore, the weighted-average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, presented based on the amounts into which the outstanding shares will convert at September 30, 2018 and 2017, respectively, from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect:

	<u>September 30,</u>	
	<u>2018</u>	<u>2017</u>
Preferred Stock	11,789,775	11,789,775
Unvested restricted stock	1,045,050	1,474,512
Options to purchase common stock	2,009,671	13,715

10. SUBSEQUENT EVENTS

In November 2018, LogicBio Australia entered into a collaboration and license agreement (the "CMRI Agreement") with Children's Medical Research Institute ("CMRI"), a private research institution, pursuant to which LogicBio Australia and CMRI will work together to develop new viral vectors over a two-year period from the effective date of the CMRI Agreement ("Research Term"). Pursuant to the CMRI Agreement, LogicBio Australia obtained an exclusive, worldwide license to make, have made, use, import, offer to sell and sell products covered by certain patent rights to the new viral vectors. As consideration for the license, LogicBio Australia will pay to CMRI royalties in the low single digits and certain milestone payments. In addition to the consideration for the license, LogicBio Australia will pay research funds to CMRI during the Research Term of \$2.0 million over the term of the CMRI Agreement.

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 the unaudited consolidated financial information and the notes thereto included in this Quarterly Report on Form 10-Q and the audited consolidated financial information and the notes thereto included in the prospectus that forms a part of our Registration Statement on Form S-1 (File No. 333-227523), which was filed with the Securities and Exchange Commission, or SEC, pursuant to Rule 424(b)(4) on October 22, 2018, or the Prospectus.

This discussion contains certain forward-looking statements that involve risks and uncertainties. Forward-looking statements are identified by words such as “believe,” “will,” “may,” “estimate,” “continue,” “anticipate,” “intend,” “should,” “plan,” “expect,” “predict,” “could,” “potentially” or the negative of these terms or similar expressions. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other “forward-looking” information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements. Factors that might cause such a difference include, but are not limited to, those discussed in the “Risk Factors” section in this Quarterly Report on Form 10-Q. Forward-looking statements are based on our management’s beliefs and assumptions and on information currently available to our management. These statements, like all statements in this Quarterly Report on Form 10-Q, speak only as of their date, and except as required by law, we undertake no obligation to update or revise these statements in light of future developments. We caution investors that our business and financial performance are subject to substantial risks and uncertainties.

Overview

We are a genome editing company focused on developing medicines to durably treat rare diseases in patients with significant unmet medical need using GeneRide, our proprietary technology platform. Our GeneRide technology is designed to precisely integrate corrective genes into a patient’s genome to provide a stable therapeutic effect. Because GeneRide is designed to have this durable therapeutic effect, we are initially targeting rare liver disorders in pediatric patients where it is critical to provide treatment early in a patient’s life before irreversible disease pathology can occur. We have demonstrated proof of concept of our therapeutic platform in animal models for a number of diseases and are focusing on development of our lead product candidate, LB-001, for the treatment of Methylmalonic Acidemia, or MMA, a life-threatening disease that presents at birth.

Based on our GeneRide technology, we are developing our lead product candidate, LB-001, to treat MMA. We expect to file an IND for LB-001 in late-2019 and initiate a Phase 1/2 trial in patients with MMA shortly thereafter. We believe that achieving clinical proof of concept in an inherited liver disease such as MMA will validate our platform technology, including its potential application to other organs and diseases. In addition to MMA, we have demonstrated proof of concept of our platform in hemophilia B, alpha-1-antitrypsin deficiency, or A1ATD, and Crigler-Najjar syndrome animal disease models. We expect to select future product candidates from these genetic diseases or others addressed by targeting the liver initially, and later by targeting the CNS and muscle.

Since our inception in 2014, we have devoted all of our efforts to business planning, research and development, developing and protecting our intellectual property, raising capital, recruiting management and technical staff. We do not have any products approved for sale and have not generated any revenue. As of September 30, 2018, we funded our operations solely from the sale of our convertible preferred stock. We have received net cash proceeds of \$33.1 million from sales of our convertible preferred stock.

[Table of Contents](#)

We have incurred significant operating losses since our inception. Our ability to generate product revenue sufficient to achieve profitability will depend on the successful development and commercialization of one or more of our product candidates. Our net losses were \$4.7 million and \$1.0 million for the three months ended September 30, 2018 and 2017, respectively, and \$10.6 million and \$2.8 million for the nine months ended September 30, 2018 and 2017, respectively. As of September 30, 2018, we had an accumulated deficit of \$20.2 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future in connection with our ongoing activities.

Furthermore, we now incur additional costs associated with operating as a public company that we did not previously incur or had previously incurred at lower rates as a private company, including significant legal, accounting, investor relations and other expenses.

Initial Public Offering

On October 23, 2018, we completed an initial public offering, or IPO, of our common stock, in which we issued and sold 8,050,000 shares of common stock, including 1,050,000 shares sold pursuant to the full exercise of the underwriters' option to purchase additional shares, at a public offering price of \$10.00 per share. The aggregate net proceeds to us from the IPO were approximately \$72.4 million after deducting underwriting discounts and commissions and offering expenses. The shares began trading on The Nasdaq Global Market on October 19, 2018. Upon completion of the IPO, all of our outstanding shares of convertible preferred stock, converted into 11,789,775 shares of our common stock.

Recent Developments

Announced Partnership with Children's Medical Research Institute to Develop Next Generation Viral Vectors

In November 2018, we announced the launch of a partnership to develop new viral vectors with Children's Medical Research Institute of Australia. This partnership will focus on developing next-generation synthetic adeno-associated virus capsids capable of overcoming many of the current limitations of existing viral vectors. We will hold exclusive worldwide commercial rights to vectors developed under the partnership.

Components of Results of Operations

Revenue

Since inception through September 30, 2018, we have not generated any revenue. We do not expect to generate any revenue from the sale of products in the near future. If our development efforts for LB-001, or other product candidates that we may develop in the future, are successful and result in regulatory approval or collaboration or license agreements with third parties, we may generate revenue in the future from a combination of product sales or payments from collaboration or license agreements.

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 product candidates, and include:

- salaries, benefits and other related costs, including stock-based compensation expense, for personnel engaged in research and development functions;
- license maintenance fees and milestone fees incurred in connection with various license agreements;
- the costs of laboratory supplies and acquiring, developing and manufacturing preclinical study and, eventually, clinical trial materials;
- expenses incurred under agreements with contract research organizations, or CROs, contract manufacturing organizations, or CMOs, as well as academic institutions and consultants that conduct our preclinical studies and other scientific development services;

Table of Contents

- facility-related expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of facilities and other operating costs;
- costs of outside consultants, including their fees, stock-based compensation and related travel expenses; and
- costs related to compliance with regulatory requirements.

We expense research and development costs as incurred. Costs for external development activities are recognized based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our financial statements as prepaid or accrued research and development expenses.

Research and development activities are central to our business model. We expect that our research and development expenses will continue to increase for the foreseeable future as we initiate clinical trials for our product candidate LB-001 and continue to discover and develop additional product candidates. If any of our product candidates enter into later stages of clinical development, they will generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in our executive, finance, corporate and business development and administrative functions. General and administrative expenses also include professional fees for legal, patent, accounting, auditing, tax and consulting services, travel expenses, and facility-related expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of facilities and other operating costs.

We expect that our general and administrative expenses will increase in the future as we increase our general and administrative headcount to support our continued research and development and potential commercialization of our product candidates. We also expect to incur increased expenses associated with being a public company, including costs of accounting, audit, legal, regulatory and tax compliance services, director and officer insurance costs; and investor and public relations costs.

Other (Expense) Income, Net

Interest income (expense), net consists primarily of interest on our cash and cash equivalents. Other (expense) income, net consists primarily of foreign exchange gains and losses.

[Table of Contents](#)**Results of Operations****Three Months Ended September 30, 2018 and 2017**

The following table summarizes our results of operations for the three months ended September 30, 2018 and 2017:

	Three Months Ended September 30,	
	2018	2017
	<i>(in thousands)</i>	
Operating expenses:		
Research and development	\$ 2,432	\$ 580
General and administrative	2,119	470
Total operating expenses	<u>4,551</u>	<u>1,050</u>
Loss from operations	<u>(4,551)</u>	<u>(1,050)</u>
Other (expense) income:		
Other (expense) income, net	<u>(80)</u>	<u>35</u>
Loss before income taxes	<u>(4,631)</u>	<u>(1,015)</u>
Income tax provision	<u>(38)</u>	<u>(29)</u>
Net loss	<u><u>\$ (4,669)</u></u>	<u><u>\$ (1,044)</u></u>

Research and Development Expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2018 and 2017:

	Three Months Ended September 30,		Increase
	2018	2017	
	<i>(in thousands)</i>		
LB-001 external development and manufacturing costs	\$ 714	\$ —	714
Personnel-related costs	720	239	481
Other research and development costs	<u>998</u>	<u>341</u>	<u>657</u>
Total research and development expenses	<u><u>\$ 2,432</u></u>	<u><u>\$ 580</u></u>	<u><u>\$ 1,852</u></u>

Research and development expenses for the three months ended September 30, 2018 were \$2.4 million, compared to \$0.6 million for the three months ended September 30, 2017. The increase of approximately \$1.9 million was primarily due to an increase of approximately \$0.7 million in other research and development expenses as we increased our overall research and development activities, \$0.5 million in personnel-related costs related to an increase in headcount, and \$0.7 million related to manufacturing expenses for our lead product candidate LB-001. Personnel-related costs for the three months ended September 30, 2018 included stock-based compensation expense of \$51,000, compared to \$41,000 for the three months ended September 30, 2017.

General and Administrative Expenses

General and administrative expenses were \$2.1 million for the three months ended September 30, 2018, compared to \$0.5 million for the three months ended September 30, 2017. The increase of approximately \$1.6 million was primarily due to legal and professional fees and personnel-related costs, including salaries, stock-based compensation and bonuses. The increase in professional fees was primarily due to the increase in legal, auditing and consulting services provided. The increase in personnel-related costs was primarily due to an increase in headcount of executive level employees. Stock-based compensation expense included in general and administrative expenses was \$0.2 million and \$7,000 for the three months ended September 30, 2018 and 2017, respectively.

Table of Contents

Other (Expense) Income, Net

Other (expense) income, net was (\$80,000) for the three months ended September 30, 2018, compared to other (expense) income, net of \$35,000 for the three months ended September 30, 2017. The change was primarily related to the loss on the sale of property and equipment.

Nine Months Ended September 30, 2018 and 2017

The following table summarizes our results of operations for the nine months ended September 30, 2018 and 2017:

	Nine Months Ended September 30,	
	2018	2017
	<i>(in thousands)</i>	
Operating expenses:		
Research and development	\$ 6,113	\$ 1,467
General and administrative	4,453	1,303
Total operating expenses	<u>10,566</u>	<u>2,770</u>
Loss from operations	<u>(10,566)</u>	<u>(2,770)</u>
Other (expense) income:		
Other (expense) income, net	44	36
Loss before income taxes	(10,522)	(2,734)
Income tax provision	(38)	(48)
Net loss	<u><u>\$(10,560)</u></u>	<u><u>\$(2,782)</u></u>

Research and Development Expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2018 and 2017:

	Nine Months Ended September 30,		Increase
	2018	2017	
	<i>(in thousands)</i>		
LB-001 external development and manufacturing costs	\$2,001	\$ —	2,001
Personnel-related costs	1,714	692	1,022
Other research and development costs	<u>2,398</u>	<u>775</u>	<u>1,623</u>
Total research and development expenses	<u><u>\$6,113</u></u>	<u><u>\$1,467</u></u>	<u><u>\$4,646</u></u>

Research and development expenses for the nine months ended September 30, 2018 were \$6.1 million, compared to \$1.5 million for the nine months ended September 30, 2017. The increase of approximately \$4.6 million was primarily due to an increase of approximately \$1.6 million in other research and development expenses as we increased our overall research and development activities, \$1.0 million in personnel-related costs related to an increase in headcount, and \$2.0 million related to manufacturing expenses for our lead product candidate LB-001. Personnel-related costs for the nine months ended September 30, 2018 and 2017 each included stock-based compensation expense of \$0.1 million.

General and Administrative Expenses

General and administrative expenses were \$4.5 million for the nine months ended September 30, 2018, compared to \$1.3 million for the nine months ended September 30, 2017. The increase of approximately \$3.2 million was primarily due to legal and professional fees and personnel-related costs, including salaries, stock-based compensation and bonuses. The increase in professional fees was primarily due to the

[Table of Contents](#)

increase of costs related to preparation for our IPO. The increase in personnel-related costs was primarily due to an increase in headcount of executive level employees. Stock-based compensation expense included in general and administrative expenses was \$0.4 million and \$30,000 for the nine months ended September 30, 2018 and 2017, respectively.

Other (Expense) Income, Net

Other (expense) income, net was \$44,000 for the nine months ended September 30, 2018, compared to other (expense) income, net of \$36,000 for the nine months ended September 30, 2017. The change was primarily related to an increase in interest income offset by the loss on the sale of property and equipment.

Liquidity and Capital Resources

Overview

Since our inception and through September 30, 2018, we have not generated any revenue and have incurred significant losses and negative cash flows from our operations.

Cash Flows

The following table summarized our cash flows for each of the nine months ended September 30, 2018 and 2017:

	Nine Months Ended	
	September 30,	
	2018	2017
	<i>(in thousands)</i>	
Net cash used in operating activities	\$(10,627)	\$ (2,957)
Net cash used in investing activities	(327)	(36)
Net cash (used in) provided by financing activities	(764)	28,703
Effect on foreign exchange rates on cash and cash equivalents	20	(26)
Net (decrease) increase in cash and cash equivalents	<u>\$(11,698)</u>	<u>\$25,684</u>

Operating Activities

During the nine months ended September 30, 2018, operating activities used \$10.6 million of cash, primarily from our net loss of \$10.6 million and net cash used by changes in our operating assets and liabilities of \$0.8 million, partially offset by non-cash charges of \$0.7 million, which were primarily related to stock-based compensation expense and loss on disposal of property and equipment. Changes in net cash in our operating assets and liabilities for the nine months ended September 30, 2018 consisted primarily of a \$0.6 million decrease in accounts payable and a \$0.6 decrease to prepaid expenses and other current assets, partially offset by a \$0.2 million increase in accrued expenses and other liabilities and a \$0.2 million increase to other non-current assets.

During the nine months ended September 30, 2017, operating activities used \$3.0 million of cash, primarily from our net loss of \$2.8 million and net cash used by changes in our operating assets and liabilities of \$0.4 million, partially offset by non-cash charges of \$0.2 million, which were primarily related to stock-based compensation expense. Changes in net cash in our operating assets and liabilities for the nine months ended September 30, 2017 consisted primarily of a \$0.3 decrease to prepaid expenses and other current assets and a \$0.2 million decrease to accrued expenses, partially offset by a \$0.1 million increase to accounts payable.

Investing Activities

During the nine months ended September 30, 2018, net cash used in investing activities was \$0.3 million, primarily from the purchase of property and equipment, partially offset by the disposal of property and equipment.

[Table of Contents](#)

During the nine months ended September 30, 2017, net cash used in investing activities was \$36,000 for the purchase of property and equipment.

Financing Activities

During the nine months ended September 30, 2018, net cash used in financing activities was \$0.8 million, primarily for the payment of deferred IPO costs, partially offset by the proceeds from the exercise of stock options.

During the nine months ended September 30, 2017, net cash provided by financing activities was \$28.7 million, primarily from net proceeds from the sale of the first tranche of Series B preferred stock in June 2017.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and clinical trials of our product candidates. We expect that our expenses will increase substantially if and as we:

- continue our current research programs and our preclinical development of any product candidates from our current research programs;
- initiate clinical trials for LB-001 and any other product candidates we identify and develop;
- seek to identify, assess, acquire and/or develop additional research programs and additional product candidates;
- seek marketing approvals for any product candidate that successfully complete clinical trials;
- develop, optimize, scale and validate a manufacturing process and analytical methods for any product candidates we may develop;
- establish and build out internal process and analytical development capabilities and research and preclinical grade production;
- obtain market acceptance of any product candidates we may develop as viable treatment options;
- address competing technological and market developments;
- maintain, expand and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- further develop our GeneRide technology platform;
- hire additional technical, quality, regulatory, clinical, scientific and commercial personnel; add operational, financial and management information systems and personnel, including personnel to support our process and product development, manufacturing and planned future commercialization efforts;
- make royalty, milestone or other payments under current and any future in-license agreements;
- establish and maintain supply chain and manufacturing relationships with third parties that can provide adequate products and services, in both amount, timing and quality, to support clinical development and the market demand for any product candidate for which we obtain regulatory and marketing approval;
- leasing and building new facilities, including offices and labs, to support organizational growth;
- validate and build-out a commercial-scale current Good Manufacturing Practices, or cGMP, manufacturing facility; and
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval.

Because of the numerous risks and uncertainties associated with the development of LB-001 and other product candidates and programs, and because the extent to which we may enter into collaborations with third parties for development of our product candidates is unknown, we are unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future funding requirements, both near and long-term, will depend on many factors, including:

Table of Contents

- the initiation, scope, progress, timing, costs and results of drug discovery, preclinical development, laboratory testing, and planned clinical trials for LB-001 and any other product candidates;
- the outcome, timing and cost of meeting regulatory requirements established by the U.S. Food and Drug Administration, or FDA, and other comparable foreign regulatory authorities, including resolving any potential clinical holds that may be imposed on us;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions;
- the achievement of milestones or occurrence of other developments that trigger payments under any of our current agreements or other agreements we may enter into;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under future collaboration agreements, if any;
- the effect of competing technological and market developments;
- the cost and timing of completion of clinical or commercial-scale manufacturing activities;
- the extent to which we in-license or acquire other products and technologies;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the cost of establishing sales, marketing and distribution capabilities for LB-001 and any other product candidates in regions where we choose to commercialize our product candidates, if approved; and
- the initiation, progress, timing and results of our commercialization of LB-001 and any other product candidates, if approved, for commercial sale.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials beyond those that we anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant trial delays due to patient enrollment or other reasons, we would be required to expend significant additional financial resources and time on the completion of clinical development. We may never succeed in obtaining regulatory approval for any of our product candidates.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to finance our cash needs through offerings of securities, private equity financing, debt financings, collaborations, government contracts or other strategic transactions. The terms of financing may adversely affect the holdings or the rights of our stockholders. If we are unable to obtain funding, we may be required to delay, limit, reduce or terminate some or all of our research and product development, product portfolio expansion or future commercialization efforts.

We will also continue to incur costs as a public company that we did not previously incur or have previously incurred at lower rates, including increased fees payable to the non-employee members of our board of directors, increased personnel costs, increased director and officer insurance premiums, audit and legal fees, investor relations fees and expenses for compliance with public company reporting requirements under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and rules implemented by the SEC and Nasdaq.

[Table of Contents](#)

Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of September 30, 2018:

	Payments Due by Period				
	Total	Less than 1 Year	1 to 3 Years	4 to 5 Years	More than 5 Years
Collaboration and research agreements (1)	58	58	—	—	—
Total	<u>\$ 58</u>	<u>\$ 58</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ —</u>

- (1) Represents license and research fees under our collaboration and research agreements. These amounts do not include any potential contingent payments, including those due upon the achievement by us of specified clinical, regulatory and commercial events, as applicable, or patent prosecution, royalty payments or license and research fees we may be required to make under license agreements we have entered into with various entities, including Stanford, the University of Texas and other research institutions. We have excluded these potential payments in the contractual obligations table because the timing and likelihood of these contingent payments are not currently known and would be difficult to predict or estimate.

We enter into contracts in the normal course of business with CROs, CMOs and other third parties for clinical trials and preclinical research studies and testing. Manufacturing commitments in the preceding table include agreements that are enforceable and legally binding on us and that specify all significant terms, including fixed or minimum quantities to be purchased; fixed, minimum or variable price provisions; and the approximate timing of the transaction. For obligations with cancellation provisions, the amounts included in the preceding table are limited to the non-cancelable portion of the agreement terms or the minimum cancellation fee.

We may incur potential contingent payments upon our achievement of clinical, regulatory and commercial milestones, as applicable, or royalty payments that we may be required to make under license agreements we have entered into with various entities pursuant to which we have in-licensed certain intellectual property. Due to the uncertainty of the achievement and timing of the events requiring payment under these agreements, the amounts to be paid by us are not fixed or determinable at this time and are excluded from the table above.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our condensed consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

There have been no significant changes to our critical accounting policies from those described in “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” included in our Prospectus.

Emerging Growth Company Status

The Jumpstart Our Business Startups Act of 2012 permits an “emerging growth company” such as us 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 irrevocably elected to “opt out” of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

[Table of Contents](#)

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements and do not have any holdings in variable interest entities.

Recently Issued Accounting Pronouncements

Refer to Note 2, in the accompanying notes to our condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q for a discussion of recent accounting pronouncements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities and foreign currency exchange rate sensitivities.

Interest Rate Sensitivity

As of September 30, 2018, we had cash and cash equivalents of \$12.9 million. Our exposure to interest rate sensitivity is impacted by changes in the underlying U.S. bank interest rates. Our surplus cash has been invested in interest-bearing savings accounts from time to time. We have not entered into investments for trading or speculative purposes. Due to the conservative nature of our investment portfolio, which is predicated on capital preservation of investments with short-term maturities, we do not believe an immediate one percentage point change in interest rates would have a material effect on the fair market value of our portfolio, and therefore we do not expect our operating results or cash flows to be significantly affected by changes in market interest rates.

As of September 30, 2018, we had no debt outstanding and are therefore not subject to interest rate risk related to debt.

Foreign Currency Exchange Risk

The functional currency of our wholly owned foreign subsidiary, LogicBio Research, is in the Israeli new shekel. Assets and liabilities of LogicBio Research are translated into United States dollars at the exchange rate in effect on the consolidated balance sheet date. Income items and expenses are translated at the average exchange rate in effect during the period. Stockholders' equity (deficit) amounts are translated based on historical exchange rates as of the date of each transaction. Unrealized translation gains and losses are recorded as a foreign currency translation adjustment, which is included in the consolidated statements of convertible preferred stock and stockholder's deficit as a component of accumulated other comprehensive loss. Adjustments that arise from exchange rate changes on transactions denominated in a currency other than the local currency are included in other (expense) income, net in the consolidated statements of operations as incurred. As of September 30, 2018, all operations had ceased for LogicBio Research.

We do not currently engage in currency hedging activities in order to reduce our currency exposure, but we may begin to do so in the future. Instruments that may be used to hedge future risks include foreign currency forward and swap contracts. These instruments may be used to selectively manage risks, but there can be no assurance that we will be fully protected against material foreign currency fluctuations.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of September 30, 2018. Our disclosure controls and procedures are designed to ensure that information we are required to disclose in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate to allow timely decisions regarding required disclosures, and is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms.

Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures as of September 30, 2018 were not effective due to the material weakness identified in fiscal year 2017 in our internal control over financial reporting process which included an ineffective control environment, including a lack of sufficient accounting personnel and personnel with financial reporting expertise, ineffective controls procedures, including those related to recognition in the appropriate period for certain transactions, ineffective risk assessment controls, including those policies and practices that would identify changes in our business practices, which could significantly impact our consolidated financial statements and system of internal controls, and ineffective monitoring of controls related to the financial close and reporting process.

Remediation Plan

We are committed and are taking steps necessary to remediate the control deficiencies that constituted the above material weakness by implementing changes to our internal control over financial reporting. Our management has performed additional analyses, reconciliations, and other post-closing procedures.

Changes in Internal Control over Financial Reporting.

Except for the remediation efforts of the previously identified material weakness as described above, there were no changes in our internal control over financial reporting that occurred during the three months ended September 30, 2018 that materially affected, or were reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may become involved in legal proceedings and claims arising in the ordinary course of our business. Although the results of litigation and claims cannot be predicted with certainty, as of the end of the period covered by this Quarterly Report on Form 10-Q, we did not believe we were party to any claim or litigation, the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors.

The following risk factors and other information included in this Quarterly Report on Form 10-Q should be carefully considered. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we currently believe to be immaterial may also adversely affect our business. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since inception and anticipate that we will incur continued losses for the foreseeable future. We may never achieve or maintain profitability.

We are a preclinical-stage genome editing company with a limited operating history. We have incurred net losses in each year since our inception, including a net loss of \$10.6 million for the nine months ended September 30, 2018. As of September 30, 2018, we had an accumulated deficit of approximately \$20.2 million. In addition, we have not commercialized any products and have never generated any revenue from product sales. We have devoted most of our financial resources to research and development, including our preclinical development activities. We expect to continue to incur significant additional operating losses for the foreseeable future as we seek to advance LB-001, our lead product candidate, through preclinical and clinical development, expand our research and development capabilities and activities, develop new product candidates, initiate and complete clinical trials, seek regulatory approval and, if we receive approval from the U.S. Food and Drug Administration, or FDA, commercialize our product candidates. Our net losses may fluctuate significantly from quarter to quarter and year to year. Because of the numerous risks and uncertainties associated with genetic medicine product development, we are unable to accurately predict the timing or amount of increased expenses, when, if ever, we will generate revenue from the commercialization of products or whether we will achieve or maintain profitability. We anticipate that our expenses will also increase substantially if and as we:

- continue our current research programs and our preclinical development of any product candidates from our current research programs;
- initiate preclinical testing and clinical trials for LB-001 and any other product candidates we identify and develop;
- seek to identify, assess, acquire and/or develop additional research programs and additional product candidates;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- develop, optimize, scale and validate a manufacturing process and analytical methods for any product candidates we may develop;

Table of Contents

- establish and build out internal process and analytical development capabilities and research and preclinical grade production;
- obtain market acceptance of any product candidates we may develop as viable treatment options;
- address competing technological and market developments;
- maintain, expand and protect our intellectual property portfolio and provide reimbursement of third-party expenses related to our patent portfolio;
- further develop our GeneRide technology platform;
- hire additional technical, quality, regulatory, clinical, scientific and commercial personnel;
- add operational, financial and management information systems and personnel, including personnel to support our process and product development, manufacturing and planned future commercialization efforts;
- make royalty, milestone or other payments under current or future in-license agreements;
- establish and maintain supply chain and manufacturing relationships with third parties that can provide adequate products and services, in both amount, timing and quality, to support clinical development and the market demand for any product candidate for which we obtain regulatory and marketing approval;
- lease and build new facilities, including offices and labs, to support organizational growth;
- validate and build-out a commercial-scale current Good Manufacturing Practices, or cGMP, manufacturing facility; and
- continue our transition operating as a public company.

Furthermore, our ability to successfully develop, commercialize and license our product candidates and potentially generate product revenue is subject to substantial additional risks and uncertainties. Each program and any product candidate we develop, will require additional preclinical and clinical development, potential regulatory approval in one or more jurisdictions, securing manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenue from product sales. See “Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval” and “Risks Related to Commercialization.”

As a result of all of the above, as well as other potential factors, we expect to continue to incur net losses and negative cash flows for the foreseeable future. These net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders’ equity and working capital. The amount of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. If we are unable to develop and commercialize one or more product candidates either alone or with collaborators, or if revenue from any product candidate that receives marketing approval is insufficient, we will not achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability.

We will require additional capital to fund our operations, and if we fail to obtain necessary financing, we may not be able to complete the development and commercialization of any product candidates.

We expect to spend substantial amounts to complete the development of, seek regulatory approvals for and commercialize LB-001 and any other product candidate we may identify and develop. We will require additional capital, which we may seek to raise through equity offerings, debt financings, marketing and distribution arrangements, collaborations, strategic alliances, licensing arrangements or other sources, to enable us to complete the development and potential commercialization of LB-001 and any other product candidate. Adequate additional financing 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 effect on our financial condition and our ability to pursue our business strategy. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities and harm our product candidate development efforts.

[Table of Contents](#)

Based upon our current operating plan, we believe that our cash and cash equivalents as of September 30, 2018, together with the net proceeds from our IPO, will enable us to fund our operating expenses and capital expenditure requirements through 2020. We anticipate that we may need additional funding in order to complete the Phase 1/2 clinical trial of LB-001. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances could cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more than currently expected because of circumstances beyond our control. Because the length of time and activities associated with successful development of LB-001 and any other product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and, if applicable, any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including:

- the initiation, scope, progress, timing, costs and results of drug discovery, preclinical development, laboratory testing, and planned clinical trials for LB-001 and any other product candidates;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA and other comparable foreign regulatory authorities, including resolving any potential clinical holds that may be imposed on us;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions;
- the achievement of milestones or occurrence of other developments that trigger payments under any of our current agreements or other agreements we may enter into;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under future collaboration agreements, if any;
- the effect of competing technological and market developments;
- the cost and timing of completion of clinical or commercial-scale manufacturing activities;
- the costs of continuing to operate as a public company;
- the extent to which we in-license or acquire other products and technologies;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the cost of establishing sales, marketing and distribution capabilities for LB-001 and any other product candidates in regions where we choose to commercialize our product candidates, if approved; and
- the initiation, progress, timing and results of our commercialization of LB-001 and any other product candidates, if approved, for commercial sale.

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 one or more are approved, we may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of medicines that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. We cannot be certain that additional funding will be available on acceptable terms, or at all. 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 LB-001 or any other product candidates or potentially discontinue operations.

[Table of Contents](#)

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

To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common 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, making capital expenditures or declaring dividends. In addition, debt financing would result in increased fixed payment obligations. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

We have never generated revenue from product sales and may never be profitable.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, product candidates we may identify for development. To date, we have not generated any revenue from our programs or any product candidate and do not anticipate generating revenues from product sales for the foreseeable future. Our ability to generate future revenues from product sales depends heavily on our, or our collaborators', ability to successfully:

- identify product candidates and complete research and preclinical and clinical development of any product candidates we may identify;
- obtain sufficient financial and other resources to complete the necessary preclinical studies and clinical trials of LB-001 and any other product candidate we may develop;
- obtain a commercial license from the United States National Institutes of Health, or NIH, for the use of the transgene in our LB-001 product candidate in clinical studies and for commercialization;
- obtain successful data from our clinical program that supports an acceptable risk-benefit profile of any product candidates in the intended populations;
- develop safe and effective delivery mechanisms for our in vivo therapeutic programs;
- achieve desirable medicinal properties for the intended indications;
- seek and obtain regulatory and marketing approvals for any product candidate for which we complete clinical trials;
- launch and commercialize any product candidate for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualify for adequate healthcare coverage and reimbursement by government and third-party payors for any product candidate for which we obtain regulatory and marketing approval;
- develop, enhance, scale and validate a manufacturing process and analytical methods for any product candidates we may develop;

Table of Contents

- implement effective strategies and knowledge management systems to ensure the integrity of data, specifically the completeness, consistency and accuracy of data used to ensure the safety, efficacy and quality of products manufactured;
- establish and maintain supply and manufacturing relationships with third parties that remain compliant with all relevant health authority and legal requirements and can provide adequate, in amount, timing and quality, products and services to support clinical development and the market demand for any product candidate for which we obtain regulatory and marketing approval;
- compete with other therapies and treatment options;
- obtain market acceptance of any product candidates we may develop as viable treatment options;
- obtain a positive recommendation from the Recombinant DNA Advisory Committee of the NIH;
- obtain a continued acceptable safety profile of the medicines following approval;
- address competing technological and market developments;
- implement internal systems and infrastructure, as needed;
- enter into collaborations to further the development of any product candidate;
- negotiate favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;
- maintain, protect and expand our portfolio of intellectual property rights, including patents, trade secrets, know-how and non-patent exclusivity for our medicines;
- avoid and defend against third-party interference or infringement claims; and
- attract, hire and retain qualified personnel.

Additionally, because our technology involves genome editing, we are subject to additional challenges and risks that gene therapy companies face, including:

- regulatory requirements that govern gene and cell therapy products, which have changed frequently and may continue to change in the future, and few products that involve the genetic modification of patient cells have been approved in the United States or the European Union; and
- the FDA's recommendation of a follow-up observation period of up to 15 years or longer for all patients who receive treatment using genome editing therapies, necessitating us to adopt such an observation period for any product candidate we may develop.

Risks Related to Discovery, Development, Clinical Testing, Manufacturing and Regulatory Approval

We intend to identify and develop product candidates based on our novel GeneRide technology platform, which makes it difficult to predict the time and cost of product candidate development. No genome editing product has been approved in the United States or in Europe. There have only been a limited number of human clinical trials involving a gene editing product candidate and none of those trials has involved our nuclease-free genome editing technology.

We have concentrated our research and development efforts on product candidates utilizing our GeneRide technology. Our future success depends on the successful development of this novel therapeutic approach. To date, no product that utilizes our GeneRide technology has been approved. There have been a limited number of clinical trials of gene editing technologies, however no product candidates have been approved, and none of these clinical trials involved product candidates that utilize our GeneRide technology. In addition, because our programs are all in the research or preclinical stage, we have not yet been able to assess safety in humans, and there may be long-term effects from treatment with any of our future product candidates that we cannot predict at this time. Any product candidates we may develop will act at the level of DNA, and, because animal DNA differs from human DNA, results of tests of our product candidates in

[Table of Contents](#)

animal models for either safety or efficacy may not be predictive of results that may be observed in humans. Also, animal models may not exist for some of the diseases we expect to pursue. Our GeneRide genome editing approach harnesses homologous recombination, or HR, a naturally occurring DNA repair process that maintains the fidelity of the genome. The mechanism of action of this technology is still not completely understood. Therefore, it is and will be difficult for us to determine whether any of our product candidates will be able to properly integrate corrective DNA in or deliver gene transfer constructs to enough tissue cells to reach therapeutic levels. We cannot be certain that any of our product candidates will be able to meet safety and efficacy levels needed to be therapeutic in humans or that they will not cause significant adverse events or toxicities. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our GeneRide technology platform, or any similar or competitive gene therapy or gene editing platforms, will result in the identification, development and regulatory approval of any medicines, or that other genetic medicine technologies will not be considered better or more attractive for the development of medicines. Any development problems we experience in the future related to our GeneRide technology platform or any of our research programs may cause significant delays or unanticipated costs, or we may not be able to solve for the issue. We may also experience delays in developing a capable and scalable manufacturing process or transferring that process to commercial partners. Any of these factors may prevent us from completing our preclinical studies or clinical trials that we may initiate or prevent us from commercializing any product candidates we may develop on a timely or profitable basis, if at all.

Because genome editing is novel and the regulatory landscape that governs any product candidates we may develop is uncertain and may change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

Because genome editing is novel, the regulatory requirements governing any genome editing product candidates we develop are uncertain and subject to change. For example, the FDA recently issued several guidance documents regarding gene therapy in July 2018. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Tissues and Advanced 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 are also subject to review and oversight by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at the institution participating in the clinical trial. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH, are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation. 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 EMA. 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 genome editing product candidates we may develop, but that remains uncertain at this point.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy products, cell therapy products or products developed through the application of gene editing technology may cause the FDA, the EMA and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing genome editing 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

[Table of Contents](#)

market of the potential products. The regulatory approval process for product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Furthermore, during the regulatory review process, we will need to identify success criteria and endpoints such that the FDA, the EMA or other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop. As we are initially seeking to identify and develop product candidates to treat diseases in which there is little clinical experience using new technologies, there is heightened risk that the FDA, the EMA or other regulatory authorities may not consider the clinical trial endpoints that we propose to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. This may be a particularly significant risk for many of the genetically defined diseases for which we plan to develop product candidates because many of these diseases have small patient populations, and designing and executing a rigorous clinical trial with appropriate statistical power is more difficult than with diseases that have larger patient populations. Further, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the European Union and other countries, such as the CAT, may make similar comments with respect to these endpoints and data. Any product candidates we may develop will be based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. No genome editing product has been approved in the United States or in Europe.

Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing genome editing 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 and development programs or the commercialization of resulting products.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, increase the scope of process development, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop.

We have no history of conducting clinical trials or commercializing genetic medicine product candidates and we may encounter difficulties transitioning from a research-stage to clinical-stage company to ultimately a commercial-stage company, which may make it difficult to evaluate the prospects for our future viability.

We are an early-stage company. We were founded in 2014 and began operations in 2015. Our operations to date have been limited to financing and staffing our company, developing our technology, identifying and developing LB-001, undertaking preclinical studies, business planning and raising capital. All of our research programs are still in the preclinical or research stage of development, and the risk of failure in the biopharmaceutical industry for programs or products candidates at such stage of development is high. We have not yet demonstrated an ability to successfully initiate, conduct or complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approval, manufacture a clinical or commercial scale product or arrange for a third party to do so on our behalf or conduct sales and marketing activities necessary for successful product commercialization. Typically, it takes about six to ten years to develop a new drug from the time it enters Phase 1 clinical trials to when it is approved for treating patients, but in many cases it may take longer. Consequently, predictions 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 product candidates.

[Table of Contents](#)

In addition, as a business with a limited operating history, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will eventually need to transition from a company with a research focus to a company capable of supporting clinical and, if any of our product candidates are approved, commercial activities. We may not be successful in such a transition.

Preclinical drug development is uncertain. Some or all of our preclinical programs may experience delays or may never advance to clinical trials, which would adversely affect our ability to obtain regulatory approvals or commercialize these product candidates on a timely basis or at all, which would have an adverse effect on our business.

In order to obtain FDA approval to market a new product, we must demonstrate proof of safety, purity and potency or efficacy in humans. To satisfy these requirements, we will have to conduct adequate and well-controlled clinical trials. Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our planned IND in the United States. We cannot be certain of the timely completion or outcome of our preclinical testing and studies, and we cannot predict if the FDA will accept our proposed clinical programs, if any, or if the outcome of our preclinical testing and studies will ultimately support the further development of any of our product candidates. As a result, we cannot be sure that we will be able to submit INDs or similar applications for any preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs or similar applications will result in the FDA or other regulatory authorities allowing clinical trials to begin.

Conducting preclinical testing is a lengthy, time-consuming and expensive process. The length of time may vary substantially according to the type, complexity, novelty and intended use of the product candidate, and often can be several years or more per product candidate. Delays associated with product candidates for which we are conducting preclinical testing and studies ourselves may cause us to incur additional operating expenses. Moreover, we may be affected by delays associated with the preclinical testing and studies of certain product candidates conducted by our potential partners over which we have no control. The commencement and rate of completion of preclinical studies and initiation of clinical trials for a product candidate may be delayed by many factors, including, for example:

- inability to generate sufficient preclinical or other in vivo or in vitro data to support the initiation of clinical trials; and
- delays in reaching a consensus with regulatory agencies on study design.

Moreover, even if we obtain positive results from preclinical studies or initial clinical trials, we may not achieve the same success in future trials.

Clinical trials are expensive, difficult to design and implement, time-consuming and involve an uncertain outcome.

Before obtaining marketing approval from regulatory authorities for the sale of any product candidates we may identify and develop, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy in humans of any such product candidates. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process, and there is a high failure rate for product candidates proceeding through clinical trials. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biotechnology and genetic medicine industries have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Even if our future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of LB-001 for MMA or any other potential indication. Our future clinical trial results may not be successful.

[Table of Contents](#)

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. 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 product candidates.

To date, we have not completed any clinical trials required for the approval of LB-001. Although we plan to initiate a Phase 1/2 clinical trial shortly after submitting our IND in late-2019, we may experience delays in conducting any clinical trials, and we do not know whether planned clinical trials will begin on time, need to be redesigned to address clinical holds imposed by regulatory authorities or for other reasons, recruit and enroll patients on time or be completed on schedule, or at all. Clinical trials can be delayed or terminated for a variety of reasons, including delays or failures related to:

- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials;
- obtaining regulatory approval to commence a trial or to restart a trial following a clinical hold;
- reaching an agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining institutional review board, or IRB, approval at each site;
- recruiting suitable patients to participate in a trial;
- developing and validating the companion diagnostic to be used in a clinical trial, if applicable;
- having patients complete a trial or return for post-treatment follow-up;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- clinical sites deviating from trial protocol or subjects dropping out of a trial;
- addressing patient safety concerns that arise during the course of a trial;
- occurrence of serious adverse events associated with any product candidates we may develop that are viewed to outweigh their potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- adding a sufficient number of clinical trial sites; or
- manufacturing sufficient quantities of product candidate for use in clinical trials.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including:

- we may receive feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon development programs;
- difficulty in designing well-controlled clinical trials due to ethical considerations which may render it inappropriate to conduct a trial with a control arm that can be effectively compared to a treatment arm;

[Table of Contents](#)

- difficulty in designing clinical trials and selecting endpoints for diseases that have not been well-studied and for which the natural history and course of the disease is poorly understood;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we or our investigators might have to suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate and we may not have funds to cover the costs;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; and
- any future collaborators that conduct clinical trials may face any of the above issues, and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for indications or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed 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 the product removed from the market after obtaining marketing approval;
- 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 sued; or
- experience damage to our reputation.

[Table of Contents](#)

We could encounter delays if a clinical trial is suspended or terminated by us, either independently or based on a recommendation by the Data Safety Monitoring Board, or DSMB, for such trial, by the IRBs of the institutions in which such trials are being conducted or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including (1) failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols; (2) inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold; (3) unforeseen safety issues or adverse side effects; (4) failure to demonstrate a benefit from using a drug; (5) changes in governmental regulations; or (6) administrative actions or lack of adequate funding to continue the clinical trial. Furthermore, we may rely on CROs and clinical trial sites to ensure the proper and timely conduct of clinical trials and while we would have agreements governing their committed activities, we would have limited influence over their actual performance, as described in “—Risks Related to Our Dependence on Third Parties.”

Our lead product candidate, LB-001, is still in preclinical development. We plan to submit an IND for LB-001 in late-2019, and we cannot provide any assurance that the FDA will authorize us to initiate any of our planned clinical trials on a timely basis, or at all, or that the FDA will agree with the design of our protocol. LB-001 will require extensive clinical testing before we are prepared to submit a biologic license application, or BLA, for regulatory approval. We cannot predict with any certainty if or when we might complete the development of LB-001 and submit a BLA for regulatory approval of LB-001 or whether any such BLA will be approved by the FDA. We may also seek feedback from the FDA or other regulatory authorities on our clinical development program, and the FDA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs.

If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of LB-001 or any other product candidate we develop could be harmed. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials also ultimately may lead to the denial of regulatory approval of our product candidates.

Product development costs also will increase if we or our collaborators experience delays in testing or marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured, or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize any product candidates we may develop, could allow our competitors to bring products to market before we do and could impair our ability to successfully commercialize any product candidates we may develop, any of which may harm our business, financial condition, results of operations and prospects.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We or our collaborators may not be able to initiate or continue clinical trials for any product candidates we identify or develop if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or analogous regulatory authorities outside the United States, or as needed to provide appropriate statistical power for a given trial. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. In addition, if patients are unwilling to participate in our genome editing trials because of negative publicity from adverse events related to the biotechnology, gene therapy or genome editing fields, competitive clinical trials for similar patient populations, clinical trials in competing products or for other reasons, the timeline for recruiting patients, conducting trials and obtaining regulatory approval of any product candidates we may develop may be delayed.

[Table of Contents](#)

We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. The enrollment of patients depends on many factors, including:

- the patient inclusion and exclusion criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- severity of the disease under investigation;
- the proximity of patients to trial sites;
- the design of the trial;
- availability and efficacy of approved medications for the disease under investigation;
- availability of genetic testing for potential patients;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new products that may be approved for the indications we are investigating;
- our ability to obtain and maintain patient consents;
- the risk that patients enrolled in clinical trials will drop out of the trials before completion;
- perceived risks and benefits of the product candidate under trial;
- perceived risks and benefits of genome editing as a therapeutic approach;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Our ability to successfully initiate, enroll, and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with CROs and physicians;
- different standards for the conduct of clinical trials;
- different standard-of-care for patients with a particular disease;
- inability to locate qualified local consultants, physicians and partners; and
- potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in such clinical trial sites.

[Table of Contents](#)

Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop LB-001 or any other product candidates, or could render further development impossible.

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

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if any product candidates we may develop meet their safety and efficacy endpoints in clinical trials, the regulatory authorities 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 authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a Risk Evaluation and Mitigation Strategy, or REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use, or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of any product candidates we may develop. Any of the foregoing scenarios could materially harm the commercial prospects for any product candidates we may develop and materially adversely affect our business, financial condition, results of operations and prospects.

Adverse public perception of genetic medicine, and gene editing in particular, may negatively impact regulatory approval of, or demand for, our potential products.

Our product candidates involve editing the human genome. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene editing and gene therapy for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene editing 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.

In addition, gene editing technology is subject to public debate and heightened regulatory scrutiny due to ethical concerns relating to the application of gene editing technology to human embryos or the human germline. For example, in April 2015, Chinese scientists reported on their attempts to edit the genome of human embryos to modify the gene for hemoglobin beta. This is the gene in which a mutation occurs in patients with the inherited blood disorder beta thalassemia. Although this research was purposefully conducted in embryos that were not viable, the work prompted calls for a moratorium or other types of restrictions on gene editing of human eggs, sperm and embryos. The Alliance for Regenerative Medicine in Washington has called for a voluntary moratorium on the use of gene editing technologies in research that involved altering human embryos or human germline cells. Similarly, the NIH has announced that it would not fund any use of gene editing technologies in human embryos, noting that there are multiple existing legislative and regulatory prohibitions against such work, including the Dickey-Wicker Amendment, which prohibits the use of appropriated funds for the creation of human embryos for research purposes or for research in which human embryos are destroyed. Laws in the United Kingdom prohibit genetically modified embryos from being implanted into women, but embryos can be altered in research labs under license from the Human Fertilisation and Embryology Authority. Research on embryos is more tightly controlled in many other European countries.

[Table of Contents](#)

Although we do not use our technologies to edit human embryos or the human germline, such public debate about the use of gene editing technologies in human embryos and heightened regulatory scrutiny could prevent or delay our development of product candidates. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair our development and commercialization of product candidates or demand for any products we may develop. Adverse events in our preclinical studies or clinical trials or those of our competitors or of academic researchers utilizing gene therapy or gene editing technologies, even if not ultimately attributable to product candidates we may discover and develop, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of potential product candidates we may identify and develop, stricter labeling requirements for those product candidates that are approved, a decrease in demand for any such product candidates and a suspension or withdrawal of approval by regulatory authorities of our product candidates.

We may not be successful in our efforts to identify additional product candidates.

Part of our strategy involves identifying novel product candidates. All of our product candidates are still in the preclinical or research stage of development. The process by which we identify product candidates may fail to yield product candidates for clinical development for a number of reasons, including:

- we may not be able to assemble sufficient resources to acquire or discover additional 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 exclusive 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;
- 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.

In addition, we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases that may later prove to have greater commercial potential or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights. If we are unable to identify additional suitable product candidates for clinical development, this could harm our business, financial condition, results of operations and prospects.

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

We currently have relationships with a limited number of suppliers for the manufacturing of our product candidates. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to transfer or sublicense the intellectual property rights we may have with respect to such activities.

[Table of Contents](#)

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including data management) 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 can impact safety, efficacy and quality, and may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's current good laboratory practices, or GLP, and cGMP regulations enforced by the FDA through its facilities inspection program. Some of our contract manufacturers have not produced a commercially-approved product and therefore have not yet obtained the requisite FDA approvals to do so. The facilities and quality systems of some or all of our third-party contractors, as well as any facilities and quality systems we may have in the future, must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates 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.

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 trial 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.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial 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 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.

Our product candidates may cause serious adverse events or undesirable side effects or have other properties that may delay or prevent their regulatory approval, limit the commercial profile of an approved label or result in significant negative consequences following marketing approval, if any.

We have not evaluated any product candidates in human clinical trials. It is impossible to predict when or if any product candidates we may develop will prove safe in humans. In the genomic 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 genome editing technologies will not cause undesirable side effects.

[Table of Contents](#)

Serious adverse events or undesirable side effects caused by any product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects, toxicities or unexpected characteristics, including death. A significant risk in many gene editing products is that the edit will be “off-target” (or “on-target,” but unwanted) and cause serious adverse events, undesirable side effects, toxicities or unexpected characteristics. For example, off-target cuts could lead to disruption of a gene or a genetic regulatory sequence at an unintended site in the DNA, or, in those instances where we also provide a segment of DNA to serve as a repair template, it is possible that following off-target cut events, DNA from such repair template could be integrated into the genome at an unintended site, potentially disrupting another important gene or genomic element. While we believe our GeneRide technology obviates this through the use of HR, we cannot be certain that off-target editing will not occur in any of our planned or future clinical trials. There is also the potential risk of delayed adverse events following exposure to gene editing therapy, due to the potential for persistent biological activity of the genetic material or other product components used to carry the genetic material. In addition to serious adverse events or side effects caused by any product candidate we may develop, the administration process or related procedures also can cause undesirable side effects. If any such events occur, our clinical trials could be suspended or terminated.

If unacceptable side effects arise in the development of any of our product candidates, we, including in consultation with the DSMB, the FDA or the IRBs at the institutions in which our studies are conducted or DSMB, could suspend or terminate our clinical trials or the FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. In that case, 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 harm our business, financial condition, results of operations and prospects. Many product candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented further clinical development of the product candidates. Treatment-related side effects also could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately recognized or managed by the treating medical staff. We expect to have to train medical personnel using our product candidates to understand the side effect profiles for our clinical trials and upon any commercialization of any of our product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in patient injury or death. Any of these occurrences may harm our business, financial condition and prospects significantly.

If in the future we are unable to demonstrate that such adverse events were caused by factors other than our product candidate, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates we are able to develop for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, 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 we may develop, 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 identify and develop product candidates, which could harm our business, financial condition, results of operations and prospects.

Additionally, if we successfully develop a product candidate and it receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits of treatment with such product candidate outweighs the risks for each potential patient. A REMS may include, among other things, a medication guide outlining the risks of the product for distribution to patients, a communication plan to health care practitioners, extensive patient monitoring or distribution systems and processes that are highly controlled, restrictive and more costly than what is typical for the industry.

[Table of Contents](#)

If any of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by any such product, including during any long-term follow-up observation period recommended or required for patients who receive treatment using our products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- we may be required to recall a product or change the way such product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product;
- regulatory authorities may require additional warnings on the label, such as a “black box” warning or contraindication;
- we may be required to implement a REMS, or create a medication guide outlining the risks of such side effects for distribution to patients;
- the product could become less competitive;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, which could harm our business, financial condition, results of operations and prospects.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable, but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the type, complexity and novelty of the product candidates involved and the substantial discretion of the regulatory authorities. In addition, approval policies, regulations or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate’s clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate and it is possible that neither LB-001 nor any other product candidate we may seek to develop in the future will ever obtain regulatory approval. Neither we nor any future collaborator is permitted to market any of our product candidates in the United States until we receive regulatory approval of a BLA from the FDA. It is possible that the FDA may refuse to accept for substantive review any BLAs that we submit for our product candidates or may conclude after review of our data that our application is insufficient to obtain marketing approval of our product candidates.

Prior to obtaining approval to commercialize a product candidate in the United States or abroad, we or our collaborators must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or foreign regulatory agencies, that such product candidates are safe and effective for their intended uses. 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. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or it may object to elements of our clinical development program. Depending on the extent of these or any other FDA-required studies, approval of any BLA or application that we submit may be delayed by several years, or may require us to expend significantly more resources than we have available.

[Table of Contents](#)

Of the large number of potential products in development, only a small percentage successfully completes the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, results of operations and prospects.

We are heavily dependent on the success of LB-001, our lead product candidate, which is still under preclinical development, and if LB-001 does not receive regulatory approval in the United States or other jurisdictions, or is not successfully commercialized, our business will be harmed.

To date, we have invested a significant portion of our efforts and financial resources in the development of LB-001. Our future success and ability to generate product revenue is substantially dependent on our ability to successfully develop, obtain regulatory approval for and successfully commercialize this product candidate. We currently have no products that are approved for commercial sale and may never be able to develop marketable products.

We expect that a substantial portion of our efforts and expenditures over the next few years will be devoted to LB-001. Accordingly, our business currently depends heavily on the successful development, regulatory approval and commercialization of LB-001, which may never occur. We cannot be certain that LB-001 will be successful in clinical trials, receive regulatory approval or be successfully commercialized even if we receive regulatory approval. Even if we receive approval to market LB-001 from the FDA or other regulatory bodies, we cannot be certain that our product candidate will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. Additionally, the research, testing, manufacturing, labeling, approval, sale, marketing and distribution of genetic medicine products are and will remain subject to extensive regulation by the FDA and other regulatory authorities in the United States and other countries that each have differing regulations.

We are not permitted to market LB-001 in the United States until it receives approval of a BLA from the FDA, or in any foreign countries until it receives the requisite approval from such countries.

We have not submitted a BLA to the FDA or comparable applications to other regulatory authorities and do not expect to be in a position to do so for the foreseeable future.

LB-001 is our lead product candidate, and because any other product candidate would be based on similar technology, if LB-001 shows unexpected adverse events or a lack of efficacy in the indications we intend to treat, or if we experience other regulatory or developmental issues, our development plans and business could be significantly harmed. Further, competitors may be developing products with similar technology and may experience problems with their products that could identify problems that would potentially harm our business.

We plan to file our Investigational New Drug application, or IND, to begin our first clinical trial for our MMA program targeting the liver in late-2019. Commencing this clinical trial, and any other clinical trials we may initiate, is also subject to finalizing the trial design based on discussions with the FDA and other regulatory authorities, including the NIH. In the event that the FDA requires us to complete additional preclinical studies or we are required to satisfy other FDA requests, the start of our first clinical trial for our MMA programs or any of our other programs 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 our 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.

[Table of Contents](#)

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by the FDA in the United States does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country.

Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on 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 product candidates that we identify for specific indications. 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. Our resource allocation decisions may cause us to fail to timely 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 products. 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 harm our business, financial condition, results of operations and prospects.

Genomic medicines are novel, and any product candidates we develop may be complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business.

Due to the novel nature of our platform, any product candidates we may develop will likely require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as the product candidates we intend to develop generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. 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 or insufficient inventory. If we successfully develop product candidates, we may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, European Union or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA, the EMA and other regulatory authorities may require us to submit samples of any lot of any 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

[Table of Contents](#)

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 clinical trials or product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

We or our third-party contractors also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to manage and/or provide the necessary oversight of our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

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 are derived from biologic sources. Such raw materials are 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.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in third-party manufacturing process or facilities also could restrict our ability to meet market demand for any products we develop and commercialize.

Our employees and independent contractors, including principal investigators, CROs, consultants, vendors and any third parties we may engage in connection with development and commercialization may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could harm our business.

Misconduct by our employees and independent contractors, including principal investigators, CROs, consultants, vendors, and any third parties we may engage in connection with development and commercialization, could include intentional, reckless or negligent conduct or unauthorized activities that violate: (i) the laws and regulations of the FDA, EMA rules and regulations and other similar regulatory requirements, including those laws that require the reporting of true, complete and accurate information to such authorities; (ii) manufacturing standards; (iii) data privacy, security, fraud and abuse and other healthcare laws and regulations; or (iv) laws that require the reporting of true, complete and accurate financial information and data. Activities subject to these laws could also involve the improper use or misrepresentation of information obtained in the course of clinical trials, creation of fraudulent data in preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, 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 governmental investigations or other actions or lawsuits stemming from a failure to comply with such laws or regulations. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. 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 and results of operations, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgements, possible exclusion from participation in Medicare, Medicaid, other U.S. federal healthcare programs or healthcare programs in other jurisdictions, individual imprisonment, additional integrity oversight and reporting obligations, other sanctions, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

[Table of Contents](#)

Our business and operations would suffer in the event of system failures.

Our computer systems, as well as those of our CROs, third-party manufacturers, suppliers and other contractors and consultants, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product candidate development programs. For example, the loss of preclinical study or clinical trial data from completed, ongoing or planned trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of personal, confidential or proprietary information, we could incur liability and the further development of LB-001 or any other product candidate could be delayed.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, clinical trial data, proprietary business information, personal data and personally identifiable information of our clinical trial subjects and employees, in our data centers and on our networks. The secure processing, maintenance and transmission of this information is critical to our operations. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or internal bad actors, or breached due to employee error, a technical vulnerability, malfeasance or other disruptions. Although, to our knowledge, we have not experienced any such material security breach to date, any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, significant regulatory penalties, and such an event could disrupt our operations, damage our reputation, and cause a loss of confidence in us and our ability to conduct clinical trials, which could adversely affect our reputation and delay our clinical development of our product candidates.

Interim “top-line” and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim “top-line” or preliminary data from our clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or “top-line” data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

Risks Related to Our Dependence on Third Parties

We currently contract with third parties for the manufacture and testing of materials. This reliance on third parties increases the risk that we will not have sufficient quantities of such materials, product candidates or any medicines that we may develop and commercialize, or that such supply will not be available to us at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We have manufacturing facilities to supply some of our discovery and preclinical research. We currently also rely on third-party manufacturers for the manufacture and some aspects of testing of our materials for preclinical studies and expect to continue to do so for clinical testing and for commercial supply of any product candidates that we may develop and for which we or our collaborators obtain marketing approval. We do not have a long term supply agreement with any of the third-party manufacturers, and we purchase our required supply on a purchase order basis. We are contracting with manufacturers that can produce the clinical and commercial supply of our product candidates.

[Table of Contents](#)

We may be unable to establish any agreements with third-party manufacturers for clinical and commercial supply manufacturing, or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the possible breach of the manufacturing agreement by the third party;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and
- reliance on the third party for regulatory compliance, quality assurance, safety, and pharmacovigilance and related reporting.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocations, seizures or recalls of product candidates or medicines, operating restrictions, and criminal prosecutions, any of which could harm our business, financial condition, results of operations and prospects.

Any medicines that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply for bulk drug substances. If any one of our current contract manufacturers cannot perform as agreed, we may be required to replace that manufacturer. Although we believe that there are a few potential alternative manufacturers who could manufacture any product candidates we may develop, we may incur added costs and delays in identifying and qualifying any such replacement.

Our current and anticipated future dependence upon others for the manufacture of any product candidates we may develop or medicines may adversely affect our future profit margins and our ability to commercialize any medicines that receive marketing approval on a timely and competitive basis.

We intend to rely on third parties to conduct, supervise and monitor our clinical trials. If those third parties do not successfully carry out their contractual duties, or if they perform in an unsatisfactory manner, it may harm our business.

We currently rely and expect to continue to rely on third parties to conduct some aspects of our research and preclinical testing. We intend to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and we expect to have limited influence over their actual performance. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it would delay our product development activities.

We intend to rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future preclinical studies. Our reliance on CROs for clinical development activities limits our control over these activities, but we will remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities. We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of any product candidates we may develop or commercialization of our medicines, producing additional losses and depriving us of potential product revenue.

[Table of Contents](#)

We and our CROs will be required to comply with the GLPs and GCPs, which are regulations and guidelines enforced by the FDA and are also required by the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities in the form of International Conference on Harmonization guidelines for any of our product candidates that are in preclinical and clinical development. The regulatory authorities enforce GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot be sure that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the regulatory approval process. We will also be required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity, and civil and criminal sanctions.

Our CROs will not be our employees, and we will not control whether or not they devote sufficient time and resources to our future clinical and preclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials, or other product development activities which could harm our competitive position. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize any product candidate that we develop. As a result, our financial results and the commercial prospects for any product candidate that we develop would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If our relationship with any CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not harm our business, financial condition, results of operations and prospects.

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 collaborative relationships for the development and commercialization of any product candidate we may develop. These collaborators could include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. 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 partly 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.

[Table of Contents](#)

Failure to obtain such collaborative relationships could impair the potential for any product candidate we may develop. We also will need to enter into collaborative relationships to provide funding to support our other research and development programs. The process of establishing and maintaining collaborative relationships is difficult, time-consuming and involves significant uncertainty, such as:

- a collaboration partner may have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- a collaboration partner may shift its priorities and resources away from our product candidates due to a change in business strategies, or a merger, acquisition, sale or downsizing;
- a collaboration partner may delay clinical trials, provide insufficient funding for a clinical trial program, stop a 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;
- a collaboration partner may seek to renegotiate or terminate its relationship with us due to unsatisfactory clinical results, manufacturing issues, a change in business strategy, a change of control or other reasons;
- a collaboration partner may cease development in therapeutic areas which are the subject of our strategic collaboration;
- a collaboration partner may not devote sufficient capital or resources towards our product candidates;
- a collaboration partner may change the success criteria for a product candidate thereby delaying or ceasing development of such candidate;
- a collaboration partner 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;
- a significant delay in initiation of certain development activities by a collaboration partner will also delay payment of milestones tied to such activities, thereby impacting our ability to fund our own activities;
- a collaboration partner could develop a product that competes, either directly or indirectly, with our product candidate;
- a collaboration partner with commercialization obligations may not commit sufficient financial or human resources to the marketing, distribution or sale of a product;
- a collaboration partner with manufacturing responsibilities may encounter regulatory, resource or quality issues and be unable to meet demand requirements;
- a collaboration partner may terminate a strategic alliance and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates;
- a dispute may arise between us and a partner concerning the research, development or commercialization of a product candidate resulting in a delay in milestones, royalty payments or termination of an alliance and possibly resulting in costly litigation or arbitration which may divert management attention and resources;
- a partner may use our products or technology in such a way as to invite litigation from a third party;
- we may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner, or at all. If a 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.

[Table of Contents](#)

If our collaborations do not result in the successful development and commercialization of products, 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 herein apply to the activities of our collaborators.

We may in the future decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of any product candidates we may develop. 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 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 any collaborator fails to fulfill its responsibilities in a timely manner, or at all, our research, clinical development, manufacturing or commercialization efforts related to that collaboration could be delayed or terminated, or it may be necessary for us to assume responsibility for expenses or activities that would otherwise have been the responsibility of our collaborator. If we are unable to successfully transition terminated collaborative agreements, we may have to delay or discontinue further development of one or more of our product candidates, undertake development and commercialization activities at our own expense or find alternative sources of capital. Moreover, any collaborative partners we enter into agreements with in the future may shift their priorities and resources away from our product candidates or seek to renegotiate or terminate their relationships with us.

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 and strategic partners 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 products or potential products that are the subject of these collaborations. 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.

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, or fail to devote sufficient resources to the development and commercialization of products. Any of these developments could harm our product development efforts.

Our collaborators or strategic partners may decide to adopt alternative technologies or may be unable to develop commercially viable products with our technology, which would negatively impact our revenues and our strategy to develop these products.

Our collaborators or strategic partners may adopt alternative technologies, which could decrease the marketability of our GeneRide technology. Additionally, because our current or future collaborators or

[Table of Contents](#)

strategic partners are likely to be working on more than one development project, they could choose to shift their resources to projects other than those they are working on with us. If they do so, this would delay our ability to test our technology and would delay or terminate the development of potential products based on our GeneRide technology. Further, our collaborators and strategic partners may elect not to develop products arising out of our collaborative and strategic partnering arrangements or to devote sufficient resources to the development, manufacturing, marketing or sale of these products. The failure to develop and commercialize a product candidate pursuant to our agreements with our current or future collaborators would prevent us from receiving future milestone and royalty payments which would negatively impact our revenues.

If we fail to comply with obligations in agreements under which we in-license or acquire development or commercialization rights to products, technology or data from third parties, including our agreements with Stanford University and the University of Texas through which we license our core technology or the license that we intend to enter into with the NIH for development and commercial rights to the transgene for LB-001, we could lose such rights that are important to our business, and we may be unable to continue our development or commercialization programs as a result, which would be harmful to our business.

We are a party to agreements with Stanford University and the University of Texas to license our core technology, and we may enter into additional agreements, including a license agreement with NIH to obtain development and commercialization rights to the transgene for LB-001, with other parties in the future that impose diligence, development and commercialization timelines, milestone payments, royalties, insurance and other obligations on us.

In exchange for the rights granted to us pursuant to the Stanford agreement and the University of Texas agreement, we are obligated to make payments upon the achievement of certain milestone events and to pay annual maintenance fees and specified royalties. If we fail to comply with our obligations under our agreements with Stanford University and the University of Texas or any future license agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market any product candidate that is covered by these agreements, which could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient patent protection for any product candidates and for our technology, or if the scope of the patent 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 commercialize our product candidates and our technology may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our platform technology and any proprietary product candidates and technology we develop. We seek to protect our proprietary position by in-licensing intellectual property relating to our platform technology and filing patent applications in the United States and abroad related to our technologies and product candidates that are important to our business. If we or our licensors are unable to obtain or maintain patent protection with respect to our AAV capsid technology and genome editing platform technology and any proprietary products and technology we develop, our business, financial condition, results of operations and prospects could be materially harmed. Additionally, if we do not adequately protect our intellectual property, our competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability.

[Table of Contents](#)

No consistent policy regarding the scope of claims allowable in the field of gene therapy has emerged in the United States. The scope of patent protection outside of the United States is also uncertain. Pending and future patent applications may not result in issued patents which protect our business, in whole or in part, or which effectively prevent others from commercializing competitive products. Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions, obtain, maintain, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents. With respect to both in-licensed and owned intellectual property, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States.

The patent prosecution process is expensive, time-consuming, and complex. We may not be able to file, prosecute, maintain, enforce, or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we, or any future partners, collaborators or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities in time to obtain patent protection. Therefore, we may miss potential opportunities to strengthen our patent position. Additionally, although we enter into agreements containing non-disclosure and confidentiality obligations with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, collaborators, contract manufacturers, consultants, and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope or patent term adjustments. If we or our partners, collaborators, licensees, or licensors, whether current or future, fail to establish, maintain, or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, licensees, or licensors are not fully cooperative or disagree with us as to the prosecution, maintenance, or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind 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 be certain that we were the first to make the inventions that will be claimed in our future patents or future patent applications, or that we will be the first to file for patent protection of such inventions. Similarly, we cannot be certain that parties from whom we do or may license or purchase patent rights were the first to make relevant claimed inventions, or were the first to file for patent protection for them. If third parties have filed patent applications on inventions claimed in our patents or applications on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs.

[Table of Contents](#)

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we license or will file issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in-license may be challenged by third parties, narrowed, circumvented, invalidated, or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical product candidates, or limit the duration of the patent protection of our product candidates. Consequently, we do not know whether any of our platform advances and product candidates will be protectable or remain protected by valid and enforceable patents. 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. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting aBLAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable, or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

Agreements through which we license patent rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. We have not had and do not have primary control over patent prosecution and maintenance for certain of the patents and patent applications we license, and therefore cannot guarantee that these patents and applications will be prosecuted in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents.

The issuance of a patent is not conclusive as to its inventorship, scope, validity, or enforceability and our patents may be challenged in the courts or patent offices in the United States and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. We or our licensors may in the future become subject to a third party pre-issuance submission of prior art to the United States Patent and Trademark Office, or the USPTO, or to other patent offices around the world. Alternately or additionally, we or our licensors may become involved in opposition, derivation, revocation, re-examination, post-grant and *inter partes* review, or interference proceedings and other similar proceedings in the United States or elsewhere, challenging our patent rights or the patent rights of others on which we rely to protect our business. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights; limit the duration of the patent protection of our technology and products; allow third parties to commercialize our technology or products 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. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us no license, develop or commercialize current or future product candidates. Moreover, we, or one of our licensors, may

[Table of Contents](#)

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 may result in loss of patent rights, loss of exclusivity, 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 product candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. Pursuant to the terms of some of our license agreements with third parties, some of our third party licensors have the right, but not the obligation in certain circumstances to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents. Even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors, and cannot guarantee that we would receive it and on what terms. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. If we cannot obtain patent protection, or enforce existing or future patents against third parties, our competitive position and our financial condition could suffer.

Moreover, some of our in-licensed patents and patent applications are, and may in the future be, co-owned with third parties. Additionally some of our future patent filings may be co-owned with third parties. If we are unable to obtain an exclusive license to any such third party co-owners' interest in such patents or patent applications, such 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. In addition, we or our licensors may need the cooperation of any such co-owners of our owned and in-licensed patents in order to enforce such patents against third parties, and such cooperation may not be provided to us or our licensors. Any of the foregoing could harm our competitive position, business, financial conditions, results of operations and prospects.

Furthermore, our owned and in-licensed patents may be subject to a reservation of rights by one or more third parties. For example, the research resulting in certain of our owned and in-licensed patent rights and technology was funded in part by the U.S. government. As a result, the U.S. government has certain rights, including march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention. For example, our licensors, including Stanford, have granted the U.S. government a non-exclusive, non-transferable, irrevocable, paid-up license to practice or have practiced for or on behalf of the United States, the inventions described in certain of our in-licensed patents and patent applications, including certain aspects of our in-licensed nuclease-free genome editing technology. If the government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. These rights may permit the U.S. government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The U.S. government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the 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 inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of any of the foregoing rights could harm our competitive position, business, financial condition, results of operations, and prospects.

Our rights to develop and commercialize our technology and product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.

We are heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our AAV capsid technology and GeneRide platform technology.

[Table of Contents](#)

These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories included in all of our licenses. For example, pursuant to our license agreements with Stanford, the licensors may, under certain circumstances, grant a license to the patents that are the subject of such license agreements to a third party. Such third party would have full rights to the patent rights that are the subject of such licenses, which could impact our competitive position and enable a third party to commercialize products similar to our future product candidates and technology. In addition, our rights to our in-licensed patents and patent applications may be dependent, in part, on inter-institutional or other operating agreements between the joint owners of such in-licensed patents and patent applications. If one or more of such joint owners breaches such inter-institutional or operating agreements, our rights to such in-licensed patents and patent applications may be adversely affected, which could harm our competitive position, business, financial conditions, results of operations and prospects.

In addition, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. For example, pursuant to our intellectual property licenses for certain patent families from Stanford, our licensors retain control of preparation, filing, prosecution, maintenance, and enforcement and defense of their patents and patent applications. Therefore, 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 prosecute, maintain, enforce, and defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any of our products that are subject of such licensed rights could be adversely affected.

Our licensors may have relied on third party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could adversely affect our competitive position, business, financial conditions, results of operations, and prospects.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We have entered into license agreements with third parties and may need to obtain additional licenses from our existing licensors and others to advance our research or allow commercialization of product candidates and technologies we may develop.

In each of our license agreements, and we expect in our future agreements, we have the right under specified conditions to bring any actions against any third party for infringing on the patents we have exclusively licensed. Certain of our license agreements also require us to meet development thresholds and other obligations to maintain the license, including establishing a set timeline for developing and commercializing products. 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;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

[Table of Contents](#)

In addition, the agreements under which we currently license intellectual property or technology from third parties are 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 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. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

If we fail to comply with our obligations under our license agreements, our licensors may have the right to terminate these license agreements, in which event we might not be able to market any product or technology that is covered by these agreements, or our licensors may convert the license to a non-exclusive license, which could adversely affect the value of the product candidate being developed under the license agreement. Termination of these license agreements or reduction or elimination of our licensed rights may also result in our having to negotiate new or reinstated licenses with less favorable terms.

In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. 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 (potentially including our competitors) to receive licenses to a portion of the intellectual property that is subject to our existing licenses. Any of these events could harm our competitive position, business, financial conditions, results of operations and prospects.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretations, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

The agreements under which we currently license intellectual property or technology from third parties are 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 financial or other obligations under the relevant agreement, including the amount, if any, that may become due and payable to our licensors in connection with any sublicense income. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. If these events were to occur, they could harm our business, financial condition, results of operations and prospects.

We may not be successful in obtaining or maintaining necessary rights to current or future product candidates in clinical studies and for commercialization, including the rights to the use of the transgene in our LB-001 product candidate, for which we currently have a non-exclusive non-clinical research license, through acquisitions and in-licenses, which could delay or prevent us from commencing clinical trials and ultimately commercializing our current or future product candidates.

Because our programs may require the use of proprietary rights by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license, or use these proprietary rights.

[Table of Contents](#)

We currently have a non-exclusive license from the NIH to an engineered methylmalonyl-CoA mutase gene, the therapeutic transgene included in LB-001, our lead product candidate, that is limited to the use of this transgene for non-clinical, research uses. We do not currently have a license to use this transgene in clinical trials or for commercial uses. In order to begin clinical trials of LB-001 and, if we receive approval, to commercialize LB-001, we will need to obtain an additional license from NIH to rights to use this transgene for these activities. Although we expect to be able to receive such a license before we anticipate beginning clinical trials for LB-001, if we are unable to obtain such a license on a timely basis, we would be delayed in initiating our clinical trials for LB-001 and, if we are unable to obtain such a license at all, we would be unable to continue development of or commercialize LB-001, and our business would be materially and adversely affected. Even if we are able to obtain such a license, we do not know the economic and other terms that will be included in the license, and such terms may not be favorable to us.

Many pharmaceutical companies, biotechnology companies, and academic institutions are competing with us in the field of genome editing technology and filing patent applications potentially relevant to our business. In order to avoid infringing these third party patents, or patents that issue from these third party patent applications, we may find it necessary or prudent to obtain licenses from such third party intellectual property holders. In addition, with respect to any patents we may co-own with third parties, we may require licenses to such co-owners' interest in such patents.

We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our technology and product candidates. 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 or at all.

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 or we may decide not to execute such option if we believe such license is not necessary to pursue our program. If we are unable or opt not to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

It is possible that we may be unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have at a reasonable cost or on reasonable terms. In that event, we may be required to expend significant time and resources to redesign our technology, product candidates, or 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 candidate, which could harm our business, financial condition, results of operations, and prospects significantly.

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

Filing, prosecuting, and defending patents in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our intellectual property rights to the same extent as the laws of the United States. In addition, our intellectual property license agreements may not always include worldwide rights. The requirements for patentability may differ in certain countries, particularly in developing countries; thus, even in countries where we do pursue patent protection, there can be no assurance that any patents will issue with claims that cover our products. Consequently, we may not be able to prevent third parties from practicing our 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

[Table of Contents](#)

other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop and market their own products and, further, may export otherwise infringing products to territories where we have patent protection or licenses but enforcement is not as strong as that in the United States or if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of certain countries, 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 of our patents, misappropriation of our other intellectual property rights, or marketing of competing products in violation of our intellectual property and proprietary rights generally.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. 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.

Agreements through which we license patent rights may not give us sufficient rights to permit us to pursue enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents (or control of enforcement or defense) of such patent rights in all relevant jurisdictions as requirements may vary.

Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts, resources and attention from other aspects of our business. Moreover, such proceedings could put our patents 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 may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. Accordingly, our efforts to enforce our intellectual property and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by governmental 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 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 licensed or future owned patents and applications. In certain circumstances, we rely on our licensing partners to pay these fees due to U.S. and non-U.S. patent agencies. The USPTO and various non-U.S. government agencies require compliance with several procedural, documentary, fee payment, and other similar provisions during the patent application process and after a patent has issued. We are also dependent on our licensors to take the necessary action to comply with these requirements with respect to some of our licensed intellectual property. In some 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 or lapse of the patent or patent application, resulting in a partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market with similar or identical products or technology, which could harm our business, financial condition, results of operations and prospects.

[Table of Contents](#)

Patent terms and data exclusivity for our product candidates may be inadequate to protect our competitive position for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension) 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 drug, a method for using it, or a method for manufacturing it may be extended. 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. The applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed. Additionally or alternatively, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States and other jurisdictions could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents.

Recent patent reform legislation in the United States, including the Leahy-Smith America Invents Act, or the America Invents Act, could increase those uncertainties and costs.

[Table of Contents](#)

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 America Invents Act, or the America Invents Act, enacted in September 2011, the United States transitioned to a first inventor to file system in which, assuming that other 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. The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to challenge the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. The America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm 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. 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, the USPTO, and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that could adversely affect our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and product candidates, we also rely on trade secrets and confidentiality agreements to protect our unpatented know-how, technology, and other proprietary information and to maintain our competitive position. With respect to our GeneRide technology platform, we consider trade secrets and know-how to be an important component of our intellectual property. Trade secrets and know-how can be difficult to protect. In particular, we anticipate that with respect to our GeneRide technology platform, these trade secrets and know-how will over time be disseminated within the industry through independent development, the publication of journal articles describing the methodology, and the movement of personnel from academic to industry scientific positions.

We seek to protect these trade secrets and other proprietary technology, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, collaborators, contract manufacturers, consultants, and other third parties. We also enter into agreements containing confidentiality and invention or patent assignment obligations with our employees and certain consultants. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. 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 such third party, or those to whom they communicate such technology or information, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor or other third party, our business and competitive position could be materially and adversely harmed.

[Table of Contents](#)

We may become involved in lawsuits to protect or enforce our patents and other intellectual property rights, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our intellectual property, such as our patents or trademarks, or the patents of our licensing partners. To counter infringement or unauthorized use, we may be required to file infringement claims. Additionally or alternatively, we may be required to defend against claims of infringement filed by third parties against us. In addition, our patents or the patents of our licensing partners may in the future become involved in inventorship, priority, or validity disputes. Filing infringement claims and countering and defending against claims regarding infringement or disputes of inventorship, priority, or validity can be expensive and time consuming and divert the time and attention of our management and scientific personnel.

If we or one of our licensors were to initiate legal proceedings against a third party, the defendant could counterclaim that such patent 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, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may in the future raise challenges to the validity of our patent claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings).

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 we or our licensing partners and the patent examiner were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology or platform, or any product candidates that we may develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations, and prospects.

In any patent infringement proceeding, there is a risk that a court may decide that a patent owned or in-licensed by us is invalid or unenforceable, in whole or in part, or may refuse to stop the other party from using the technology at issue on the grounds that our owned and in-licensed patents do not cover the technology in question. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in any litigation or proceeding could put one or more of our owned or in-licensed patents at risk of being invalidated or interpreted narrowly and may curtail or preclude our ability to assert those patents against third parties and exclude third parties from making and selling similar or competitive products. Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy.

Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our personnel from their normal responsibilities. The monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of shares 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. Moreover, there can be no assurance that we will have sufficient

[Table of Contents](#)

financial or other resources to conduct such litigation or proceedings adequately, which can last for years before they are concluded. Some of our competitors 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.

The intellectual property landscape around genome editing technology is highly dynamic, and third parties may initiate legal proceedings alleging that we are infringing, misappropriating, or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could adversely affect the success of our business.

Our commercial success depends, in part, 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 and proprietary rights of third parties. However, the biotechnology and pharmaceutical industries are characterized by extensive litigation regarding patents and other intellectual property rights, and our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Moreover, due to the intense research and development that is taking place by several companies, including us and our competitors, in the field of gene therapy, the intellectual property landscape is in flux, and it may remain uncertain for the coming years. There may be significant intellectual property related litigation and proceedings relating to our owned and in-licensed, and other third party, intellectual property, and proprietary rights in the future. We may in the future become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our technology and any product candidates we may develop, including interference proceedings, post-grant review, *inter partes* review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions such as oppositions before the EPO. We cannot provide any assurances that third party patents do not exist which might be enforced against our current technology, manufacturing methods, product candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Third parties may have U.S. and non-U.S. issued patents and pending patent applications relating to products and methods of use for the treatment of the disease indications for which we are developing our product candidates. There may be third-party patents or patent applications with claims to materials, methods of manufacture, or methods for treatment related to the use or manufacture of our technologies and product candidates. If we are not able to obtain or maintain a license on commercially reasonable terms to any third-party patents that cover our product candidates or activities, such third parties could potentially assert infringement claims against us, which could harm our business.

Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may be accused of infringing. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Accordingly, third parties may assert infringement claims against us based on existing or future intellectual property rights. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects. If any third-party patents or patent applications are found to cover our product candidates or their methods of use, we may not be free to manufacture or market our product candidates as planned without obtaining a license.

[Table of Contents](#)

Even if we believe third-party intellectual property claims are without merit, there is no assurance that a court would find in our favor on questions of infringement, validity, enforceability, or priority. A court of competent jurisdiction could hold that these third party patents are valid, enforceable, and infringed, which could materially and adversely affect our ability to commercialize any product candidates we may develop and any other product candidates or technologies covered by the asserted third party patents.

The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform.

If we were sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the asserted patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence as to the invalidity of any such U.S. patent claim to overcome the presumption of validity enjoyed by issued patents. There is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, and we are unsuccessful in demonstrating that such patents are invalid or unenforceable, we could be required to obtain a license from such third party to continue developing, manufacturing, and marketing any product candidates we may develop and our technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. Alternatively or additionally, it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. We also could be forced, including by court order, to cease developing, manufacturing, and commercializing the infringing technology or product candidates. In addition, we could be found liable for 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 commercializing our product candidates 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 adverse effect on our business, financial condition, results of operations, and prospects.

We may be subject to claims that we, 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 and our licensors' employees, consultants, and advisors are currently or were previously employed at universities or other biotechnology or pharmaceutical companies, some of which may be our competitors or potential competitors. Some of these individuals executed agreements containing proprietary rights, non-disclosure and non-competition obligations, or similar agreements, in connection with such current or previous employment. 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 we or these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against these claims.

[Table of Contents](#)

In addition, while it is our policy to require our employees and consultants 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. 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.

If we fail in prosecuting or defending any such claims listed above, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our management and scientific personnel.

We may be subject to claims challenging the inventorship of our patents and other intellectual property.

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 patents, 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. 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 patents, 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 our product candidates. 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.

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 or utilize similar gene therapy technology but that are not covered by the claims of the patents that we license or may own in the future;
- others may be able to make or utilize gene therapy technology that functions as a viable alternative to technology we may develop or technology covered by the claims of the patents that we license or may own 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 license or may own 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 our owned or licensed intellectual property rights;
- it is possible that our pending licensed patent applications or those that we may own in the future 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 our competitors;

[Table of Contents](#)

- our competitors might conduct research and development activities in countries where we do not have patent 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 of others may harm our business; and
- we may choose not to file a patent in order to maintain 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 harm our business, financial condition, results of operations, and prospects.

Risks Related to Healthcare Laws and Other Legal Compliance Matters

Healthcare legislative reform measures and constraints on the national budget for social security systems may harm our business and results of operations.

Third-party payors, whether domestic or foreign, or governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell any products profitably. In particular, in 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or the Affordable Care Act, was enacted, which, among other things, subjected biologic products to potential competition by lower-cost biosimilars, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees for certain branded prescription drugs and provided incentives to programs that increase the federal government’s comparative effectiveness research.

Some of the provisions of the Affordable Care Act have yet to be implemented, while certain provisions have been subject to legal and political challenges. Since January 2017, President Trump has signed two executive orders and other directives designed to delay, circumvent or loosen certain requirements mandated by the Affordable Care Act. In December 2017, Congress repealed the tax penalty for an individual’s failure to maintain Affordable Care Act-mandated health insurance as part of a tax reform bill. Further, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain Affordable Care Act-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Moreover, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the Affordable Care Act, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” Congress could consider additional legislation to repeal or replace elements of the Affordable Care Act, or adopt other healthcare reform measures. We cannot predict the ultimate content, timing or effect of any healthcare reform legislation. Thus, the full impact of the Affordable Care Act, any law replacing elements of it, and the political uncertainty surrounding any repeal or replacement legislation on our business remains unclear.

In addition, other legislative changes have been proposed and adopted in the United States since the Affordable Care Act 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. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013, and will remain in effect through 2027 unless additional

[Table of Contents](#)

Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012, was signed into law, which, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for our drug product candidates, if we obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

Any denial in coverage or reduction in reimbursement from Medicare or other government programs may result in a similar denial or reduction in payments from private payors, which may adversely affect our future profitability.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties and other sanctions.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under U.S. federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the U.S. federal false claims and civil monetary penalties laws, including the civil False Claims Act, which, among other things, impose criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or 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. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

[Table of Contents](#)

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and its implementing regulations, which also imposes certain obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without appropriate authorization by covered entities subject to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information;
- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the U.S. Public Health Service Act, which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;
- the U.S. federal legislation commonly referred to as the Physician Payments Sunshine Act, enacted as part of the Affordable Care Act, and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children’s Health Insurance Program to report annually to the CMS information related to certain payments and other transfers of value to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- analogous U.S. state laws and regulations, including: state anti-kickback and false claims laws, which may apply to claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare professionals and entities and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; state and local laws that require the registration of pharmaceutical sales representatives; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts; and
- similar healthcare laws and regulations in the European Union and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance 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 the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, additional integrity reporting and oversight obligations, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations, any of which could harm our business. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

[Table of Contents](#)

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. Infringement of these laws could result in substantial fines and imprisonment. Payments made to physicians in certain European Union Member States must be publically disclosed. 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 not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment, which could affect our ability to operate our business.

We are subject to environmental, health and safety laws and regulations, and we may become exposed to liability and substantial expenses in connection with environmental compliance or remediation activities.

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 our current or past facilities and 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 harm 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 harm our reputation, business, financial condition, results of operations, and prospects.

[Table of Contents](#)

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 harm our reputation, business, financial condition, results of operations and prospects.

Risks Related to Employee Matters and Managing Growth

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

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the development, regulatory, commercialization and business development expertise of Fred Chereau, our President and Chief Executive Officer, and Dean Falb, our Chief Scientific Officer, as well as the other members of our management, technical, scientific, clinical and regulatory teams. Although we have formal employment agreements with our executive officers, these agreements do not prevent them from terminating their employment with us at any time. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

If we lose one or more of our executive officers or key employees, our ability to implement our business strategy successfully could be seriously harmed. 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 develop, gain regulatory approval of and commercialize product candidates successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional 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, technical and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific, technical, clinical and regulatory advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be engaged by entities other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to develop and commercialize product candidates will be limited.

We expect to grow our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of September 30, 2018, we had 23 employees. We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of product candidate development, technical development, clinical and regulatory affairs and sales, marketing and distribution. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities to devote time to managing these growth activities. To manage these growth activities, 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. Our inability to effectively manage the expansion of our operations may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our expected growth, our expenses may increase more than expected, our potential ability to generate revenue could be reduced and we may not be able to implement our business strategy.

[Table of Contents](#)

We may engage in acquisitions that could disrupt our business, cause dilution to our stockholders or reduce our financial resources.

In the future, we may enter into transactions to acquire other businesses, products or technologies. If we do identify suitable candidates, we may not be able to make such acquisitions on favorable terms, or at all. Any acquisitions we make may not strengthen our competitive position, and these transactions may be viewed negatively by customers or investors. We may decide to incur debt in connection with an acquisition or issue our common stock or other equity securities to the stockholders of the acquired company, which would reduce the percentage ownership of our existing stockholders. We could incur losses resulting from undiscovered liabilities of the acquired business that are not covered by the indemnification we may obtain from the seller. In addition, we may not be able to successfully integrate the acquired personnel, technologies and operations into our existing business in an effective, timely and nondisruptive manner. Acquisitions may also divert management attention from day-to-day responsibilities, increase our expenses and reduce our cash available for operations and other uses. We cannot predict the number, timing or size of future acquisitions or the effect that any such transactions might have on our operating results.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and may face an even greater risk if we commercialize any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any product candidates that we may develop; and
- injury to our reputation and significant negative media attention.

Our insurance coverage may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidate. 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.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our manufacturing facilities, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could harm our business.

Risks Related to Commercialization

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

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

Our focus is the development of genetic medicines. There are a number of companies developing nuclease-based gene editing technologies, including CRISPR Therapeutics, Editas Medicine, Intellia Therapeutics and Sangamo Therapeutics. We may also compete with companies developing gene therapy products, including Homology Medicines, Audentes Therapeutics, bluebird bio, uniQure and Voyager Therapeutics. Any products we may develop could also face competition from other products approved to treat the same disease based on other types of therapies, such as small molecule, antibody or protein therapies. There are several companies developing competing products that target MMA, the indication for which we are developing LB-001. These companies include Moderna Therapeutics with an mRNA based approach, Selecta Biosciences with AAV gene therapy, and Hemoshear Therapeutics using a small molecule. While none of these companies have clinical-stage programs for these therapies, any of them may obtain regulatory approval for a treatment for MMA before LB-001, which could negatively affect our ability to successfully commercialize LB-001, if approved.

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

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

[Table of Contents](#)

The successful commercialization of our product candidates will depend in part on the extent to which governmental authorities and health insurers establish coverage, adequate reimbursement levels and pricing policies. Failure to obtain or maintain coverage and adequate reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate revenue.

The availability and adequacy of coverage and reimbursement by third-party payors, including governmental healthcare programs such as Medicare and Medicaid, managed care organizations and private health insurers are essential for most patients to be able to afford prescription medications such as our product candidates, assuming FDA approval. Our ability to achieve acceptable levels of coverage and reimbursement for products by third-party payors will have an effect on our ability to successfully commercialize our product candidates. We cannot be sure that coverage and reimbursement in the United States, the European Union or elsewhere will be available for our product candidates or any product that we may develop, and any reimbursement that may become available may be decreased or eliminated in the future.

Third-party payors increasingly are challenging prices charged for pharmaceutical products and services, and many third-party payors may refuse to provide coverage and reimbursement for particular drugs or biologics when an equivalent generic drug, biosimilar or a less expensive therapy is available. It is possible that a third-party payor may consider our product candidates as substitutable and only offer to reimburse patients for the less expensive product. Even if we show improved efficacy or improved convenience of administration with our product candidates, pricing of existing third-party therapeutics may limit the amount we will be able to charge for our product candidates. These payors may deny or revoke the reimbursement status of a given product or establish prices for new or existing marketed products at levels that are too low to enable us to realize an appropriate return on our investment in our product candidates. Assuming we obtain coverage for our product candidates by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Interim reimbursement levels for new medicines, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Net prices for medicines may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of medicines from countries where they may be sold at lower prices than in the United States. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize our product candidates, and may not be able to obtain a satisfactory financial return on our product candidates.

There is significant uncertainty related to the insurance coverage and reimbursement of newly-approved products. The regulations that govern marketing approvals, pricing, and reimbursement for new medicines vary widely from country to country. In the United States, third-party payors, play an important role in determining the extent to which new drugs and biologics will be covered. The Medicare and Medicaid programs increasingly are used as models in the United States for how third-party payors develop their coverage and reimbursement policies for drugs and biologics. Some third-party payors may require pre-approval of coverage for new or innovative devices or drug therapies before they will reimburse healthcare providers who use such therapies. We cannot predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

No uniform policy for coverage and reimbursement for products exists among third-party payors in the United States. Therefore, coverage and reimbursement for products can differ significantly from payor to payor and coverage and reimbursement by one payor does not guarantee coverage and reimbursement by another payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Furthermore, rules and regulations regarding reimbursement change frequently, in some cases on short notice, and we believe that changes in these rules and regulations are likely.

[Table of Contents](#)

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 and other countries have and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medical 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 our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially-reasonable revenue and profits. Furthermore, some countries require approval of the sale price of a medicine before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a medicine in a particular country, but then be subject to price regulations that delay our commercial launch of the medicine, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the medicine in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Due to the novel nature of our technology and the potential for any product candidates we may develop to offer therapeutic benefit in a single administration or limited number of administrations, we face uncertainty related to pricing and reimbursement for these product candidates.

Our initial target patient populations are relatively small, and, as a result, the pricing and reimbursement of any product candidates we may develop, if approved, must be adequate to support the necessary commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell any such product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to any product candidates we may develop (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our products. In addition, it may be necessary for us to develop new reimbursement models in order to realize adequate value. Third-party payors may not be able or willing to adopt such new models, and patients may be unable to afford that portion of the cost that such models may require them to bear. If we determine such new models are necessary but we are unsuccessful in developing them, or if such models are not adopted by payors, our business, financial condition, results of operations and prospects could be adversely affected.

We expect the cost of a single administration of genomic medicine products, such as those we are seeking to develop, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by third-party payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of any such product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of any product candidates we may develop 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 payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

[Table of Contents](#)

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize any product candidates we may develop. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

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

We focus our research and product development on treatments for rare pediatric diseases. 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 products, or may become increasingly difficult to identify or gain access to, all of which could harm our business, financial condition, results of operations, and prospects.

If we obtain approval to commercialize any products outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If LB-001 or any other product candidate we may develop is approved for commercialization, it may be marketed in certain jurisdictions outside the United States. We expect that we will be subject to additional risks related to international pharmaceutical operations, including:

- economic weakness, including inflation, or political instability in particular non-U.S. economies and markets;
- different regulatory requirements for drug and biologic approvals and rules governing drug and biologic commercialization in foreign countries;
- reduced protection for intellectual property rights;
- difficulties in compliance with non-U.S. laws and regulations;
- changes in non-U.S. regulations and customs, tariffs and trade barriers;
- changes in non-U.S. currency exchange rates and currency controls;
- changes in a specific country's or region's political or economic environment;
- trade protection measures, import or export licensing requirements or other restrictive actions by U.S. or non-U.S. governments;
- negative consequences from changes in tax laws;
- foreign reimbursement, pricing and insurance regimes;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- difficulties associated with staffing and managing international operations, including differing labor relations;

[Table of Contents](#)

- potential noncompliance with the U.S. Foreign Corrupt Practices Act of 1977, as amended, or the FCPA, the U.K. Bribery Act 2010 and similar anti-bribery and anticorruption laws in other jurisdictions;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism, or natural disasters including floods and fires.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by both the European Union and many of the individual countries in Europe with which we will need to comply. Many U.S.-based biotechnology companies have found the process of marketing their own products in Europe to be very challenging.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws, and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. §201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, contractors, and other collaborators from authorizing, promising, offering, or providing, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties to sell our products outside the United States, to conduct clinical trials, and/or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, contractors, and other collaborators, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

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

The Affordable Care Act 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 adversely affect the future commercial prospects for our biological products.

[Table of Contents](#)

There is a risk that any of our product candidates 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 our product candidates 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 non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

Risks Related to Our Common Stock

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

Our stock price may be volatile. The stock market in general, and the market for pharmaceutical and biopharmaceutical companies in particular, has experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of companies whose stock is experiencing those price and volume limitations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual performance. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price at which they purchased them. The market price for our common stock may be influenced by many factors, including:

- the success of competitive products or technologies;
- actual or expected changes in our growth rate relative to our competitors;
- timing and results of clinical trials of any product candidate we may develop or those of our competitors;
- developments related to our collaborations;
- regulatory actions with respect to any product candidate we may develop or our competitors' products and product candidates;
- regulatory or legal developments in the United States and other countries;
- development of new product candidates that may address our markets and make our product candidates less attractive;
- failure or discontinuation of any of our product development and research programs;
- changes in physician, hospital or healthcare provider practices that may make our product candidates less useful;
- announcements by us, our partners or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- failure to meet or exceed financial estimates and projections of the investment community or that we provide to the public;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;

Table of Contents

- results of preclinical studies, clinical trials or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- actual or expected changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results, development timelines, or recommendations by securities analysts, or those of companies that are perceived to be similar to us;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders, or other stockholders;
- limited public float;
- expiration of market stand-off or lock-up agreement;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- developments or changing views regarding the use of genomic medicines, including those that involve genome editing;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section and others beyond our control.

Our principal stockholders and management own a significant percentage of our stock and are able to exercise significant influence over matters subject to stockholder approval.

As of September 30, 2018, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock, together with their respective affiliates, beneficially owned approximately 90% of our common stock, including shares subject to outstanding options that are exercisable within 60 days after such date. Upon consummation of our initial public offering, or IPO, our executive officers and directors, combined with our stockholders who owned more than 5% of our common stock before our IPO, together with their respective affiliates, continued to beneficially hold 81% of our outstanding common stock. Accordingly, these stockholders are able to exert a significant degree of influence over our management and affairs and over matters requiring stockholder approval, including the election of our board of directors and approval of significant corporate transactions. This concentration of ownership could have the effect of entrenching our management or board of directors, delaying or preventing a change in control or otherwise discouraging a potential acquirer from attempting to obtain control of us, which in turn could have a material and adverse effect on the fair market value of our common stock.

A significant portion of our total outstanding shares are eligible to 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 September 30, 2018, 15,010,900 shares of our common stock were restricted as a result of securities laws or lock-up agreements, but will become eligible to be sold at various times beginning 180 days after the date of our IPO, unless held by one of our affiliates, in which case the resale of those securities will be subject to volume limitations under Rule 144 of the Securities Act of 1933, as amended, or Rule 144. Moreover, holders of an aggregate of 11,789,775 shares of our common stock 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, until such shares can otherwise be sold without restriction under Rule 144 or until the rights terminate pursuant to

[Table of Contents](#)

the terms of the investors' rights agreement between us and such holders. We also plan to register all 3,392,885 shares of common stock that we may issue under our equity compensation plans or that are issuable upon the exercise of outstanding options. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

We are an “emerging growth 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,” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company until the last day of the fiscal year following the fifth anniversary of the closing of our IPO. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. However, if certain events occur prior to the end of such five-year period, including if we become a “large accelerated filer,” our annual gross revenues exceed \$1.07 billion or we issue more than \$1.0 billion of non-convertible debt in any three-year period, we will cease to be an emerging growth company prior to the end of such five-year period. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, 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 golden parachute payments.

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 reduced or more volatile. Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected to “opt out” of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance, or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

We are incurring significant increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives and corporate governance practices.

As a newly public company, we are incurring significant legal, accounting and other expenses that we did not 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 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.

[Table of Contents](#)

We expect that we will need to hire additional accounting, finance and other personnel in connection with our efforts of continuing to comply with the requirements of being a public company, and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. Moreover, these rules and regulations have increased and will continue to increase our legal and financial compliance costs and make some activities more time-consuming and costly.

Pursuant to SOX Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. However, while we remain an emerging growth 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 SOX Section 404 within the prescribed period, we will be 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, potentially engage outside consultants, 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 whether such 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 SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We have identified material weaknesses in our internal control over financial reporting. If we are unable to successfully remediate these material weaknesses in our internal control over financial reporting, it could have an adverse effect on our company.

We have identified certain material weaknesses in our internal control over financial reporting. These material weaknesses include: (1) an ineffective control environment, including a lack of sufficient accounting personnel and personnel with financial reporting expertise; (2) ineffective controls procedures, including those related to recognition in the appropriate period for certain transactions; (3) ineffective risk assessment controls, including those policies and practices that would identify changes in our business practices, which could significantly impact our consolidated financial statements and system of internal controls; and (4) ineffective monitoring of controls related to the financial close and reporting process. Had we performed an evaluation of our internal control over financial reporting in accordance with Section 404, additional control deficiencies may have been identified by management, and those control deficiencies could have also represented one or more material weaknesses.

In an effort to remediate these material weaknesses, we have begun hiring additional accounting and finance personnel. We have also retained an accounting consulting firm to provide additional depth and breadth in our technical accounting and financial reporting capabilities. We intend to continue this arrangement until additional permanent technical accounting resources are identified and hired. We intend to formalize our policies and procedures surrounding our financial close, financial reporting and other accounting processes. We intend to further develop and document necessary policies and procedures regarding our internal control over financial reporting, such that we are able to perform a Section 404 analysis of our internal control over financial reporting when and as required. We are in the process of recruiting additional qualified accounting and finance personnel to provide needed levels of expertise in our internal accounting function. We cannot be sure that these measures will significantly improve or remediate the material weaknesses described above. We also cannot be sure that we have identified all or that we will not have additional material weaknesses in the future. Accordingly, material weaknesses may still exist when we report on the effectiveness of our internal control over financial reporting for purposes of our attestation when required by reporting requirements under the Exchange Act or Section 404. Further, while we remain an emerging growth 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.

[Table of Contents](#)

We expect to incur additional costs to remediate these control deficiencies, though there can be no assurance that our efforts will be successful or avoid potential future material weaknesses. If we are unable to successfully remediate our existing or any future material weaknesses in our internal control over financial reporting, or if we identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our stock price may decline as a result. We also could become subject to investigations by Nasdaq, the SEC or other regulatory authorities.

If securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our stock, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. We do not currently have, and may never obtain, research coverage by securities and industry analysts. If no or few securities or industry analysts commence coverage of us, the trading price for our stock would be negatively impacted. In the event we obtain securities or industry analyst coverage, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these analysts ceases coverage of us or fails to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

Provisions in our restated certificate of incorporation and restated by-laws 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 management.

Provisions in our restated certificate of incorporation and our restated by-laws 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, our restated certificate of incorporation and our restated by-laws include provisions that:

- authorize “blank check” preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- provide that our directors may be removed only for cause;

[Table of Contents](#)

- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorized our board of directors to modify, alter or repeal our amended and restated by-laws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated by-laws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, 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.

The existence of the foregoing provisions and anti-takeover measures could limit the price that investors might be willing to pay in the future for shares of our common stock. They could also deter potential acquirers of our company, thereby reducing the likelihood that our stockholders could receive a premium for their common stock in an acquisition.

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

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, any future credit facility may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. As a result, capital appreciation, if any, of our common stock would be the sole source of gain on an investment in our common stock for the foreseeable future.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

Recent U.S. tax legislation may materially adversely affect our financial condition, results of operations and cash flows.

Recently-enacted U.S. tax legislation has significantly changed the U.S. federal income taxation of U.S. corporations, including by reducing the U.S. corporate income tax rate, limiting interest deductions, and revising the rules governing net operating losses. Many of these changes are effective immediately, without any transition periods or grandfathering for existing transactions. The legislation is unclear in many respects and could be subject to potential amendments and technical corrections, as well as interpretations and implementing regulations by the Treasury and Internal Revenue Service, any of which could lessen or increase certain adverse impacts of the legislation. In addition, it is unclear how these U.S. federal income tax changes will affect state and local taxation, which often uses U.S. federal taxable income as a starting point for computing state and local tax liabilities.

The reduction of the corporate tax rate under the legislation may cause a reduction in the economic benefit of our net operating loss carryforwards and other deferred tax assets available to us. Furthermore, under the legislation, although the treatment of tax losses generated before December 31, 2017 has generally not changed, tax losses generated in calendar year 2018 and beyond will only be able to offset 80% of taxable income. This change may require us to pay U.S. federal income taxes in future years despite generating a loss for federal income tax purposes in prior years.

[Table of Contents](#)

While some of the changes made by the tax legislation may adversely affect the Company in one or more reporting periods and prospectively, other changes may be beneficial on a going forward basis. We continue to work with our tax advisors to determine the full impact that the recent tax legislation as a whole will have on us. We urge our investors to consult with their legal and tax advisors with respect to such legislation.

If we fail to establish and maintain proper and effective internal control over financial reporting, our operating results and our ability to operate our business could be harmed.

Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that needs to be re-evaluated frequently. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. We have begun the process of documenting, reviewing and improving our internal controls and procedures for compliance with SOX Section 404, which will require annual management assessment of the effectiveness of our internal control over financial reporting. We have begun recruiting additional finance and accounting personnel with certain skill sets that we need as a public company.

Implementing any appropriate changes to our internal controls may distract our officers and employees, entail substantial costs to modify our existing processes and take significant time to complete. These changes may not, however, be effective in maintaining the adequacy of our internal controls, and any failure to maintain that adequacy, or consequent inability to produce accurate financial statements on a timely basis, could increase our operating costs and harm our business. In addition, investors' perceptions that our internal controls are inadequate or that we are unable to produce accurate financial statements on a timely basis may harm our common share price and make it more difficult for us to effectively market and sell our service to new and existing customers.

Our certificate of incorporation and bylaws designate the state or federal courts in the State of Delaware and the federal district courts of the United States, respectively, as the exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our certificate of incorporation provides that, unless our board of directors otherwise determines, the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, will be the sole and exclusive forum for 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 or officers to our company or our stockholders, any action asserting a claim against us or any of our directors or officers arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or by-laws, or any action asserting a claim against us or any of our directors or officers governed by the internal affairs doctrine. Additionally, our bylaws provide that the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action 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 or officers, which may discourage such lawsuits against us and our directors and officers. Additionally, it is unclear whether a court would enforce the exclusive forum provision in our bylaws and, if a court were to find either choice of forum provision to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such actions in other jurisdictions, which could adversely affect our results of operations and financial condition.

[Table of Contents](#)

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

Unregistered Sales of Equity Securities

In July 2018, pursuant to the anti-dilution right under our license agreement with The Board of Trustees of the Leland Stanford Junior University (“Stanford”), we issued 56,097 shares of our common stock to Stanford. No underwriters were used in the transaction and the sale of securities was made in reliance on the exemption from registration provided by Section 4(a)(2) of the Securities Act for transactions by an issuer not involving a public offering.

Use of Proceeds From Registered Securities

On October 23, 2018, we closed our IPO, in which we issued and sold 8,050,000 shares of our common stock, including 1,050,000 shares sold pursuant to the full exercise of the underwriters’ option to purchase additional shares, at a public offering price of \$10.00 per share for gross proceeds of \$80.5 million, before deducting underwriting discounts and commissions and offering expenses payable by us. 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. 333-227523), which was declared effective by the SEC on October 18, 2018. Jefferies LLC, Barclays Capital Inc. and William Blair & Company, L.L.C. acted as joint book-running managers of the offering and as representatives of the underwriters. Chardan Capital Markets, LLC acted as the lead manager for the offering. The offering commenced on October 18, 2018 and did not terminate until the sale of all of the shares offered.

The net offering proceeds to us, after deducting underwriting discounts and offering costs payable by us of an aggregate of approximately \$8.1 million, were approximately \$72.4 million. No material offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning 10.0% or more of any class of our equity securities or to any other affiliates.

We had not used any of the net offering proceeds as of September 30, 2018, as the IPO closed on October 23, 2018. There has been no material change in our planned use of the net proceeds from our IPO as described in our final prospectus filed with the SEC pursuant to Rule 424(b)(4) on October 18, 2018.

Item 6. Exhibits.

EXHIBIT 3.1	—	Fourth Amended and Restated Certificate of Incorporation (incorporated by reference to Exhibit 3.1 to the Company’s Current Report on Form 8-K, filed on October 29, 2018).
EXHIBIT 3.2	—	Amended and Restated Bylaws (incorporated by reference to Exhibit 3.2 to the Company’s Current Report on Form 8-K, filed on October 29, 2018).
EXHIBIT 31.1	—	Rule 13a—14(a) / 15d—14(a) Certifications — Chief Executive Officer.
EXHIBIT 31.2	—	Rule 13a—14(a) / 15d—14(a) Certifications — Chief Financial Officer.
EXHIBIT 32.1	—	Section 1350 Certifications.
EXHIBIT 101.INS	—	XBRL Instance Document.
EXHIBIT 101.SCH	—	XBRL Taxonomy Extension Schema Document.
EXHIBIT 101.CAL	—	XBRL Taxonomy Extension Calculation Linkbase Document.
EXHIBIT 101.DEF	—	XBRL Taxonomy Extension Definition Linkbase Document.
EXHIBIT 101.LAB	—	XBRL Taxonomy Extension Label Linkbase Document.
EXHIBIT 101.PRE	—	XBRL Taxonomy Extension Presentation Linkbase Document.

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.

Dated: December 3, 2018

LogicBio Therapeutics, Inc.

By: /s/ Frederic Chereau
Frederic Chereau
President and Chief Executive Officer

Dated: December 3, 2018

By: /s/ Matthias Jaffé
Matthias Jaffé
Chief Financial Officer

**Certification of Principal Executive Officer pursuant to Exchange Act Rules 13a-14(a)
and 15d-14(a), as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002**

I, Frederic Chereau, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of LogicBio Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ Frederic Chereau

Frederic Chereau
President and Chief Executive Officer

Dated: December 3, 2018

**Certification of Principal Financial Officer pursuant to Exchange Act Rules 13a-14(a)
and 15d-14(a), as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002**

I, Matthias Jaffé, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of LogicBio Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) 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(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ Matthias Jaffé

Matthias Jaffé
Chief Financial Officer

Dated: December 3, 2018

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

In connection with the Quarterly Report on Form 10-Q of LogicBio Therapeutics, Inc. (the "Company") for the quarter ended September 30, 2018 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the company, hereby certifies, pursuant to Section 1350 of Chapter 63 of Title 18, United States Code, that to the best of his knowledge:

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

Dated: December 3, 2018

/s/ Frederic Chereau
Frederic Chereau
President and Chief Executive Officer

Dated: December 3, 2018

/s/ Matthias Jaffé
Matthias Jaffé
Chief Financial Officer