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, 2021

OR

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

For the transition period from _____ to _____

Commission File Number: 001-38707

LogicBio Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-1514975**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
65 Hayden Avenue, 2nd Floor, Lexington, MA	**02421**
(Address of principal executive offices)	**(Zip code)**

(617) 245-0399
(Registrant's telephone number, including area code)

n/a
(Former name, former address and formal fiscal year, if changed since last report)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	**LOGC**	**The Nasdaq Global Market**

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

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

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

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

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

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

As of November 8, 2021, the registrant had 32,956,794 shares of common stock, $0.0001 par value per share, outstanding.

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NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements. All statements other than statements of historical fact are "forward-looking statements" for purposes of this Quarterly Report on Form 10-Q. In some cases, you can identify forward-looking statements by terminology such as "anticipate," "approach," "believe," "continue," "could," "designed," "estimate," "expect," "goal," "intend," "may," "plan," "potential," "project," "strategy," "will," "would," "should," "seek," "likely," "become," "develop," "engage," "execute," "expand," "goal," "leverage," "future," "vision" or similar expressions, or the negative or plural of these words or expressions. These forward-looking statements may include statements concerning the following:

- the design, cost, initiation, timing, progress and results of our current and future research and development activities, including statements with respect to our Phase 1/2 SUNRISE clinical trial and other development activities for our product candidate, LB-001, in methylmalonic acidemia, or MMA;
- early clinical results and the significance and interpretation thereof and the expected timing of announcing additional interim clinical data in the SUNRISE trial;
- potential attributes and benefits of our GeneRide™ and sAAVy™ platforms and our existing or future product candidates, including any potential benefit of such platforms over competing platforms;
- our plans to continue to enroll patients in our SUNRISE trial;
- the direct or indirect impacts of the COVID-19 pandemic on our business, operations and the markets and communities in which we and our partners, collaborators and vendors operate;
- 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 our collaboration and license agreements and our ability to enter into future collaboration and licensing arrangements;
- the timing of, and our ability to obtain and maintain, regulatory approvals for our existing or future product candidates;
- our ability to quickly and efficiently identify and develop additional product candidates;
- our ability to obtain the funding for our operations necessary to continue the advancement of any product candidates;
- our ability to advance any product candidate into and successfully complete clinical trials;
- our intellectual property position, including obtaining and maintaining patents, the duration of our patent protection and trade secret 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.

Any or all of these forward-looking statements in this Quarterly Report on Form 10-Q may turn out to be inaccurate. These forward-looking statements involve risks and uncertainties, including those that are discussed below under the heading "Risk Factors Summary", and the risk factors identified further in Part II, Item 1A. "Risk Factors" included in this Quarterly Report on Form 10-Q and elsewhere in this Quarterly Report on Form 10-Q, that could cause our actual results, financial condition, performance or achievements to be materially different from those indicated in these forward-looking statements. In particular, the impact of the ongoing COVID-19 pandemic on our ability to progress with our research, development, manufacturing and regulatory efforts, including our plans to advance and complete our Phase 1/2 SUNRISE clinical trial for LB-001 in MMA, and the value of and market for our common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the United States and in other countries, and the effectiveness of actions taken globally to contain and treat the disease. In addition we are subject to the following risks: existing preclinical data may not be predictive of the results of ongoing or later clinical trials; clinical trials may not be successful or may be discontinued or delayed for any reason; manufacturing and process development risks, including delays relating to continuously improving our manufacturing processes; risks associated with management and key personnel changes and transitional periods; the actual funding required to develop and commercialize product candidates, including for safety, tolerability, enrollment, manufacturing or economic reasons; the timing and content of decisions made by regulatory authorities; the actual time it takes to initiate and complete preclinical and clinical studies; the competitive landscape; changes in our economic and financial conditions; and our ability to obtain, maintain and enforce patent and other intellectual property protection for LB-001 and any other product candidates. Given these risks and uncertainties, you should not place undue reliance on these forward-looking statements. Forward-looking statements speak only as of the date of this Quarterly Report on Form 10-Q. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason. Unless otherwise stated, our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

In 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.

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RISK FACTORS SUMMARY

The following is a summary of the principal risks that could adversely affect our business, financial condition and results of operations:

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.
- Under our ASC 205-40 analysis, there is "substantial doubt" that we will have sufficient funds to satisfy our obligations through the next twelve months from the date of issuance of this Quarterly Report on Form 10-Q.
- 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.

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

- We intend to identify and develop product candidates based on our novel GeneRide and sAAVy technology platforms, which makes it difficult to predict the time and cost of product candidate development.
- Because gene delivery 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.
- 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.
- Clinical trials are expensive, time-consuming, difficult to design and implement and involve an uncertain outcome.
- If we encounter difficulties enrolling patients in our clinical trials, particularly in light of the COVID-19 pandemic, our clinical development activities could be delayed or otherwise adversely affected.
- 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.
- 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 may not be successful in our efforts to identify additional product candidates.
- 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.
- We are heavily dependent on the success of LB-001 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.
- 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.

Risks Related to Our Dependence on Third Parties

- Reliance on third-party manufacturers increases the risk that we will not have sufficient quantities of testing 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.
- If the third parties that conduct, supervise and monitor our clinical trials do not successfully carry out their contractual duties, or if they perform in an unsatisfactory manner, it may harm our business.

- Collaborations we enter into with third parties for the research, development and commercialization of certain of our product candidates may not be successful, we may not be able to capitalize on the market potential of those product candidates.
- 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.
- 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, or Stanford, and the University of Texas through which we license our core technology or our agreement 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.

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, our competitors could develop and commercialize products and technology similar or identical to ours.
- 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.
- Patent terms and market exclusivity for our product candidates may be inadequate to protect our competitive position for an adequate amount of time.
- 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.
- We may be subject to claims challenging the inventorship of our patents and other intellectual property.

Risks Related to Healthcare Laws and Other Legal Compliance Matters

- Our current and future business operations are and will be subject to applicable healthcare regulatory laws, which could expose us to penalties and other sanctions.
- We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies and contractual obligations could adversely affect our business.

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.
- We may encounter difficulties in managing our growth, which could disrupt our operations.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements (Unaudited).

LogicBio Therapeutics, Inc.

Condensed Consolidated Balance Sheets
(In thousands, except share and per share data)

	September 30, 2021	December 31, 2020
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 59,581	$ 70,075
Accounts receivable	119	263
Prepaid expenses and other current assets	985	2,205
Total current assets	60,685	72,543
Property and equipment, net	2,028	1,815
Restricted cash	622	622
Operating lease right-of-use asset	4,851	5,660
TOTAL ASSETS	$ 68,186	$ 80,640
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 903	$ 447
Accrued expenses and other current liabilities	3,466	2,701
Operating lease liabilities	1,193	1,094
Current portion of long-term debt	3,291	1,910
Current portion of deferred revenue	4,837	—
Total current liabilities	13,690	6,152
Long-term debt, net of issuance costs and discount	5,790	8,109
Operating lease liabilities, net of current portion	4,046	4,952
Deferred revenue, net of current portion	5,896	—
Total liabilities	29,422	19,213
COMMITMENTS AND CONTINGENCIES (Note 14)		
STOCKHOLDERS' EQUITY:		
Preferred stock, par value of $0.0001 per share; 25,000,000 shares authorized; no shares issued and outstanding as of September 30, 2021 and December 31, 2020.	—	—
Common stock, par value of $0.0001 per share; 175,000,000 shares authorized; 32,945,616 and 31,775,748 shares issued and outstanding as of September 30, 2021 and December 31, 2020, respectively	3	3
Additional paid-in capital	169,718	161,415
Accumulated other comprehensive income	—	—
Accumulated deficit	(130,957)	(99,991)
Total stockholders' equity	38,764	61,427
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 68,186	$ 80,640

See notes to unaudited condensed consolidated financial statements.

LogicBio Therapeutics, Inc.

Condensed Consolidated Statements of Operations
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
REVENUE				
Collaboration and service revenue	$ 2,120	$ 926	$ 3,383	$ 2,912
Total revenue	2,120	926	3,383	2,912
OPERATING EXPENSES				
Research and development	7,806	5,492	21,482	18,560
General and administrative	4,257	3,200	12,081	9,421
Total operating expenses	12,063	8,692	33,563	27,981
LOSS FROM OPERATIONS	(9,943)	(7,766)	(30,180)	(25,069)
OTHER INCOME (EXPENSE):				
Interest income	3	2	13	179
Interest expense	(270)	(276)	(824)	(821)
Other (expense) income, net	(3)	1	(3)	(10)
Total other expense, net	(270)	(273)	(814)	(652)
Loss before income taxes	(10,213)	(8,039)	(30,994)	(25,721)
Income tax benefit	28	—	28	—
Net loss	$ (10,185)	$ (8,039)	$ (30,966)	$ (25,721)
Net loss per share—basic and diluted	$ (0.31)	$ (0.34)	$ (0.96)	$ (1.10)
Weighted-average common stock outstanding—basic and diluted	32,443,960	23,599,052	32,181,912	23,367,804

See notes to unaudited condensed consolidated financial statements.

LogicBio Therapeutics, Inc.

Condensed Consolidated Statements of Comprehensive Loss
(In thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Net loss	$ (10,185)	$ (8,039)	$ (30,966)	$ (25,721)
Other comprehensive income:				
Unrealized gain (loss) on investments	—	—	—	—
Foreign currency translation adjustment	—	—	—	—
Comprehensive loss	$ (10,185)	$ (8,039)	$ (30,966)	$ (25,721)

See notes to unaudited condensed consolidated financial statements.

LogicBio Therapeutics, Inc.

Condensed Consolidated Statements of Stockholders' Equity
(In thousands, except share and per share data)

	Common Stock $0.0001 Par Value		Additional Paid-in	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
BALANCE, January 1, 2020	23,036,943	$ 3	$ 109,640	$ 14	$ (67,370)	$ 42,287
Vesting of restricted stock	160,340	—	—	—	—	—
Exercise of options	19,378	—	84	—	—	84
Realized gain on investments	—	—	—	(14)	—	(14)
Stock-based compensation expense	—	—	805	—	—	805
Net loss	—	—	—	—	(9,455)	(9,455)
BALANCE, March 31, 2020	23,216,661	3	110,529	—	(76,825)	33,707
Vesting of restricted stock	18,642	—	—	—	—	—
Issuance of common stock related to at-the-market offerings, net of issuance costs of $33	269,540	—	1,907	—	—	1,907
Stock-based compensation expense	—	—	769	—	—	769
Net loss	—	—	—	—	(8,227)	(8,227)
BALANCE, June 30, 2020	23,504,843	3	113,205	—	(85,052)	28,156
Vesting of restricted stock	13,381	—	—	—	—	—
Issuance of common stock related to at-the-market offerings, net of issuance costs of $41	166,937	—	1,320	—	—	1,320
Stock-based compensation expense	—	—	854	—	—	854
Net loss	—	—	—	—	(8,039)	(8,039)
BALANCE, September 30, 2020	23,685,161	$ 3	$ 115,379	$ —	$ (93,091)	$ 22,291
BALANCE, January 1, 2021	31,775,748	$ 3	$ 161,415	$ —	$ (99,991)	$ 61,427
Vesting of restricted stock	31,372	—	—	—	—	—
Issuance of common stock related to at-the-market offerings, net of issuance costs of $65	251,086	—	2,091	—	—	2,091
Stock-based compensation expense	—	—	989	—	—	989
Net loss	—	—	—	—	(10,282)	(10,282)
BALANCE, March 31, 2021	32,058,206	3	164,495	—	(110,273)	54,225
Vesting of restricted stock	84,384	—	—	—	—	—
Exercise of options	70,620	—	52	—	—	52
Issuance of common stock related to at-the-market offerings, net of issuance costs of $1	9,156	—	45	—	—	45
Stock-based compensation expense	—	—	997	—	—	997
Net loss	—	—	—	—	(10,499)	(10,499)
BALANCE, June 30, 2021	32,222,366	3	165,589	—	(120,772)	44,820
Vesting of restricted stock	11,489	—	—	—	—	—
Exercise of options	49,926		35			35
Issuance of common stock, net of issuance costs of $92	661,835	—	2,962	—	—	2,962
Stock-based compensation expense	—	—	1,132	—	—	1,132
Net loss	—	—	—	—	(10,185)	(10,185)
BALANCE, September 30, 2021	32,945,616	$ 3	$ 169,718	$ —	$ (130,957)	$ 38,764

See notes to unaudited condensed consolidated financial statements.

LogicBio Therapeutics, Inc.

Condensed Consolidated Statements of Cash Flows
(In thousands)

	Nine Months Ended September 30,	
	2021	2020
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (30,966)	$ (25,721)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	451	349
Net amortization of premiums and discounts on investments	—	26
Stock-based compensation expense	3,118	2,428
Non-cash interest expense	173	156
Non-cash lease expense	811	1,366
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	1,220	917
Accounts receivable	144	—
Accounts payable	456	411
Accrued expenses and other current liabilities	26	(1,368)
Deferred revenue	10,733	—
Net cash used in operating activities	(13,834)	(21,436)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Maturities of investments	—	17,500
Purchase of property and equipment	(734)	(343)
Net cash (used in) provided by investing activities	(734)	17,157
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercise of stock options	87	84
Net proceeds from at-the-market common stock issuances	5,098	3,227
Principal repayments on term loan	(1,111)	—
Net cash provided by financing activities	4,074	3,311
NET DECREASE IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH	(10,494)	(968)
Cash, cash equivalents and restricted cash at beginning of year	70,697	33,875
Cash, cash equivalents and restricted cash at end of period	$ 60,203	$ 32,907
RECONCILIATION OF CASH, CASH EQUIVALENTS AND RESTRICTED CASH		
Cash and cash equivalents	$ 59,581	$ 32,285
Long-term restricted cash	622	622
Total cash, cash equivalents and restricted cash	$ 60,203	$ 32,907
SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION:		
Cash paid for interest	$ 651	$ 666
SUPPLEMENTAL DISCLOSURE OF NON-CASH ACTIVITIES:		
Right-of-use assets obtained in exchange for operating lease obligation	$ —	$ 6,428
Property and equipment purchases in accrued expenses and accounts payable	$ 48	$ —
Deferred financing costs in accounts payable and accrued expenses	$ —	$ 133

See notes to unaudited condensed consolidated financial statements.

1. NATURE OF BUSINESS AND BASIS OF PRESENTATION

Business Overview

LogicBio Therapeutics, Inc. (“LogicBio” or the “Company”) was incorporated in 2014 as a Delaware corporation. Its principal offices are in Lexington, Massachusetts. LogicBio is a clinical-stage genetic medicine company pioneering gene editing and gene delivery platforms to address rare and serious diseases from infancy through adulthood. The Company's gene editing platform, GeneRide™, is a new approach to precise gene insertion harnessing a cell's natural deoxyribonucleic acid (“DNA”) repair process potentially leading to durable therapeutic protein expression levels. The Company's gene delivery platform, sAAVy™, is an adeno-associated virus (“AAV”) capsid engineering platform designed to optimize gene delivery for treatments in a broad range of indications and tissues.

Based on the Company’s GeneRide technology, LogicBio is developing its lead product candidate, LB-001, to treat methylmalonic acidemia (“MMA”) in pediatric patients. The SUNRISE trial is a multi-center, open label, Phase 1/2 clinical trial designed to assess the safety, tolerability and preliminary efficacy of LB-001 in pediatric patients with MMA characterized by methylmalonyl CoA mutase gene (“MMUT”) mutations. The Company expects seven centers in the United States and one center in Saudi Arabia to participate in the SUNRISE trial.

In April 2021, the Company entered into an Exclusive Research Collaboration, License and Option Agreement with CANbridge Care Pharma Hong Kong Limited (“CANbridge”), pursuant to which LogicBio granted CANbridge (a) an exclusive worldwide license to certain of the Company’s intellectual property rights, including those relating to AAV sL65 (“sL65”), the first capsid produced from the sAAVy platform, to develop, manufacture and commercialize gene therapy candidates for the treatment of Fabry and Pompe diseases, (b) an option to obtain an exclusive worldwide license to certain of the Company’s intellectual property rights, including those relating to sL65, to develop and commercialize gene therapy candidates for the treatment of two additional indications, and (c) an exclusive option to obtain an exclusive license to develop and commercialize LB-001 for the treatment of MMA in China, Taiwan, Hong Kong and Macau. Also in April 2021, the Company announced a research collaboration with Daiichi Sankyo Company, Limited (“Daiichi”) for the development of treatments for two indications based on GeneRide. In addition, the Company entered into a research collaboration with Takeda Pharmaceutical Company Limited (“Takeda”) in January 2020 to develop LB-301, an investigational therapy leveraging GeneRide, for the treatment of Crigler-Najjar syndrome (“CN”), a rare pediatric disease. As of September 30, 2021, the work under the research plan has been completed.

Since its inception, the Company has devoted the majority of its efforts to research and development, including its preclinical and clinical development activities and its manufacturing and process development activities, raising capital, and providing general and administrative support for these operations. 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 product candidates, successfully commercialize its products, if approved, generate revenue and attain profitable operations.

COVID-19 Impact

The Company is closely monitoring the COVID-19 pandemic in order to promote the safety of its personnel and to continue advancing its research and development activities. The Company is following federal, state and local requirements and guidelines with respect to the COVID-19 pandemic, and has allowed its employees to return to working on-premises in accordance with those requirements and guidelines.

The COVID-19 pandemic did not have a material impact on the Company’s results of operations, cash flow and financial position as of and for the nine months ended September 30, 2021. However, the Company is aware that certain of its third-party vendors are being affected by import/export and other restrictions due to COVID-19, which are currently having an impact on certain of the Company’s research, development and manufacturing activities. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company’s business, results of operations and financial position will depend on future developments that are uncertain and cannot be accurately predicted.

Liquidity and Capital Resources

The Company has had recurring losses since inception and incurred a loss of $30,966 during the nine months ended September 30, 2021. Net cash used in operations for the nine months ended September 30, 2021 was $13,834. The Company expects to continue to generate operating losses and use cash in operations for the foreseeable future. As of September 30, 2021, the Company had cash and cash equivalents of $59,581 which management believes will be sufficient to fund its operating expenses and capital expenditure requirements into the fourth quarter of 2022. On a quarterly basis, the Company is required to conduct an accounting analysis under ASC 205-40, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, or ASC 205-40. The result of the Company's ASC 205-40 analysis is that there is substantial doubt about the Company's ability to continue as a going concern through the next twelve months from the date of issuance of this Quarterly Report on Form 10-Q.

The Company will require substantial additional capital to fund its research and development, including its preclinical and clinical development activities and its manufacturing and process development activities, and ongoing operating expenses. Management's plans to mitigate the conditions that raise substantial doubt include raising additional capital through equity or debt financings, payments from its collaborators, strategic transactions, or a combination of those approaches. These plans may also include the possible deferral of certain operating expenses unless and until additional capital is received. There can be no assurance that the Company will be successful in raising additional capital or that such capital, if available, will be on terms that are acceptable to the Company, or that the Company will be successful in deferring certain operating expenses.

The accompanying condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The condensed consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty.

Basis of Presentation

The accompanying condensed consolidated financial statements 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 U.S. 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 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2020, filed with the SEC on March 15, 2021.

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, 2021, consolidated results of operations for the three and nine months ended September 30, 2021 and 2020 and cash flows for the nine months ended September 30, 2021 and 2020. Such adjustments are of a normal and recurring nature. The results of operations for the three and nine months ended September 30, 2021 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2021.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Significant Accounting Policies

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

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("FASB") or other standard setting bodies that are adopted by the Company as of the specified effective date. The Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on the Company's consolidated financial statements upon adoption.

In March 2020, the FASB issued Accounting Standards Update ("ASU") 2020-04, *Facilitation of the Effects of Reference Rate Reform on Financial Reporting (Topic 848)*. This ASU provides optional expedients and exceptions for applying U.S. GAAP to transactions affected by reference rate (e.g., LIBOR) reform if certain criteria are met, for a limited period of time to ease the potential burden in accounting for (or recognizing the effects of) reference rate reform on financial reporting. The ASU is effective as of March 12, 2020 through December 31, 2022. The Company will evaluate transactions or contract modifications, including any related to its July 2019 loan and security agreement which uses LIBOR as a reference rate, occurring as a result of reference rate reform and determine whether to apply the optional guidance on an ongoing basis. The ASU is currently not expected to have a material impact on the Company's condensed consolidated financial statements and related disclosures.

3. FAIR VALUE MEASUREMENTS

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

Description	September 30, 2021	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Other Observable Inputs (Level 3)
Assets				
Money market funds and other cash equivalents	$ 59,199	$ 59,199	$ —	$ —
Total financial assets	$ 59,199	$ 59,199	$ —	$ —

Description	December 31, 2020	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Other Observable Inputs (Level 3)
Assets				
Money market funds and other cash equivalents	$ 69,277	$ 69,277	$ —	$ —
Total financial assets	$ 69,277	$ 69,277	$ —	$ —

When developing fair value estimates, the Company maximizes the use of observable inputs and minimizes the use of unobservable inputs. When available, the Company uses quoted market prices to measure fair value. The valuation technique used to measure fair value for the Company's Level 1 and Level 2 assets is a market approach, using prices and other relevant information generated by market transactions involving identical or comparable assets. If market prices are not available, the fair value measurement is based on models that use primarily market-based parameters including yield curves, volatilities, credit ratings and currency rates. In certain cases where market rate assumptions are not available, the Company is required to make judgments about assumptions market participants would use to estimate the fair value of a financial instrument.

The Company did not have any transfers of assets between levels of the fair value measurement hierarchy during the nine months ended September 30, 2021.

4. INVESTMENTS

As of September 30, 2021 and December 31, 2020, the Company did not hold any short-term or long-term investments.

5. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other current liabilities at September 30, 2021 and December 31, 2020 consisted of the following:

	September 30, 2021	December 31, 2020
Accrued compensation and benefits	$ 1,847	$ 1,200
Accrued professional services	1,251	988
Accrued lab supplies	215	71
Accrued IP licensing fees	—	70
Other	153	372
Total accrued expenses and other current liabilities	$ 3,466	$ 2,701

Accrued compensation and benefits consists primarily of accrued bonuses. Accrued professional services consists primarily of consulting services, legal services and services provided by contract research organizations ("CRO") and contract manufacturing organizations ("CMO"). Accrued lab supplies consists primarily of reagents and lab consumables. Accrued IP licensing fees consist of fees payable to certain of the Company's existing licensors.

6. DEBT

On July 2, 2019 (the "Closing Date"), the Company entered into a loan and security agreement (the "Loan Agreement"), for term loans with Oxford Finance LLC ("Oxford") and Horizon Technology Finance Corporation ("Horizon," and, together with Oxford, the "Lenders"). The Loan Agreement allows the Company to borrow up to $20,000 issuable in two equal tranches (the "Term Loans"). On the Closing Date, the first tranche of $10,000 was drawn down by the Company (the "Term A Loan"). In September 2020 and March 2021, the Company entered into amendments to the Loan Agreement, each of which extended the availability of the $10,000 second tranche subject to certain conditions. In the second quarter of 2021, the Company met the conditions to initiate drawdown of the second tranche but did not exercise its right to do so, and the option to draw down the second tranche of the Term Loans expired.

The outstanding balance of the Term Loans will accrue interest at the greater of (i) the rate of the one-month U.S. LIBOR rate plus 6.25% and (ii) 8.75%. The Loan Agreement provides for an interest only period until July 1, 2021, followed by thirty-six equal monthly payments of principal and interest continuing through June 1, 2024 (the "Maturity Date"). The Company has the option to prepay the outstanding balance prior to the Maturity Date, subject to a prepayment fee of 1.0% to 3.0% depending upon when the prepayment occurs. Upon repayment of the Term Loans, the Company is required to make a final payment to the Lenders equal to 4.5% of the original principal amount of the Term Loans funded which will be accrued by charges to interest expense over the term of the loans using the effective interest method.

In conjunction with the Loan Agreement, the Company issued 15,686 of common stock warrants ("Warrants") to the Lenders at a per share exercise price of $12.75, a maximum contractual term of 10 years and exercisable immediately. The fair value of the Warrants was accounted for as a debt discount and calculated to be approximately $136 using the Black-Scholes method. The Company determined the Warrants met the criteria for equity classification, and, as such, the fair value of the Warrants is recorded as additional paid-in capital on the condensed consolidated balance sheets. Finally, the Company incurred issuance costs of approximately $150. Both the debt discount and issuance costs will be accreted to long-term debt on the condensed consolidated balance sheets by charges to interest expense over the term of the Term A Loan using the effective interest method.

The Loan Agreement contains customary representations, warranties and covenants and also includes customary events of default. Events of default include, among other things, the Company's failure to pay amounts due, a breach of certain covenants, a material adverse change event, misrepresentations and judgments. Upon the occurrence of an event of default, a default interest rate of an additional 5.00% per annum may be applied to the outstanding loan balances, and the Lenders may declare all outstanding obligations immediately due and payable. Borrowings under the Loan Agreement are collateralized by substantially all the Company's assets, other than its intellectual property, which include maintaining certain cash balances in controlled accounts.

Interest expense was $270 and $824 for the three and nine months ended September 30, 2021, respectively. Interest expense was $276 and $821 for the three and nine months ended September 30, 2020, respectively. The effective interest rate on the Loan Agreement, including the amortization of the debt discount and issuance costs, and accretion of the final payment, was 11.34% at September 30, 2021. The components of the long-term debt balance are as follows:

	September 30, 2021	December 31, 2020
Notes payable, gross	$ 8,889	$ 10,000
Less: Unamortized debt discount and issuance costs	(112)	(175)
Accretion of final payment fee	304	194
Carrying value of notes payable	9,081	10,019
Less: Current portion of long-term debt	(3,291)	(1,910)
Long-term debt, net of issuance costs and discount	$ 5,790	$ 8,109

As of September 30, 2021, the estimated future principal payments due were as follows:

	As of September 30, 2021
2021	$ 834
2022	3,333
2023	3,333
2024	1,389
Thereafter	—
Total principal payments	$ 8,889

7. STOCK-BASED COMPENSATION

Equity Incentive Plans

In December 2014, the Company adopted the LogicBio Therapeutics, Inc. 2014 Equity Incentive Plan, as amended (the "2014 Plan"), for the issuance of stock options and other stock-based awards. In October 2018, the Company's 2018 Equity Incentive Plan (the "2018 Plan") became effective and as a result, no further awards will be made under the 2014 Plan. The 2018 Plan was established to provide equity-based ownership opportunities for employees and directors, as well as outside consultants and advisors. Any awards granted under the 2014 Plan prior to the adoption of the 2018 Plan remained outstanding in accordance with their respective terms.

Under the 2018 Plan, there is an annual increase on January 1 of each year from 2019 until 2028, by the lesser of (i) 4% of the number of shares of common stock outstanding on December 31 of the prior year and (ii) an amount determined by the Company's Board of Directors ("Board"). On January 1, 2021, the Company increased the number of shares available for future grant under the 2018 Plan by 1,272,547 shares. At September 30, 2021, there were 759,002 shares available for future grant under the 2018 Plan.

The 2018 Plan is administered by the Compensation Committee of the Board, except with respect to such matters that are not delegated to the Compensation Committee by the Board (collectively, the "Administrator"). The exercise prices, vesting and certain other restrictions are determined at the discretion of the Administrator, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the common stock on the date of grant. The term of stock options awarded under the 2018 Plan may not exceed 10 years from the grant date. Stock options, shares of restricted stock and restricted stock units ("RSU") granted to employees, officers, members of the Board, advisors, and consultants of the Company typically vest over one to four years.

Stock Options

During the nine months ended September 30, 2021 and 2020, the Company granted options with time-based vesting to purchase 2,097,256 and 870,203 shares of common stock, respectively, with a weighted-average grant date fair value per share of $4.13 and $4.80, respectively. The Company recorded stock-based compensation expense for options granted of $2,812 and $1,999 during the nine months ended September 30, 2021 and 2020, respectively. As of September 30, 2021, there were 4,508,640 outstanding options, of which 2,693,368 were unvested, and $9,738 of unrecognized stock-based compensation expense to be recognized over a weighted-average period of 2.9 years.

Restricted Common Stock

The Company has granted shares of restricted common stock with time-based and performance-based vesting conditions from time to time. The Company did not grant any restricted common stock during the nine months ended September 30, 2021 or 2020. The Company recorded stock-based compensation expense for restricted common stock granted of $132 and $108 during the nine months ended September 30, 2021 and 2020, respectively. As of September 30, 2021, there were 11,153 shares of unvested restricted common stock outstanding, all of which have been expensed as of September 30, 2021.

Restricted Stock Units

The Company has granted RSUs with time-based vesting conditions from time to time. Each RSU represents the right to receive one share of the Company's common stock upon vesting. The fair value is calculated based upon the Company's closing stock price on the date of grant, and the stock-based compensation expense is recognized over the vesting period. The Company granted 5,939 and 125,737 RSUs during the nine months ended September 30, 2021 and 2020, respectively. The Company recorded stock-based compensation for RSUs granted of $174 and $321 during the nine months ended September 30, 2021 and 2020, respectively. As of September 30, 2021, there were 5,939 unvested RSUs outstanding and $14 of unrecognized stock-based compensation expense related to unvested RSUs to be recognized over a weighted-average period of 0.3 years.

Stock-Based Compensation Expense

Total stock-based compensation expense recorded as research and development and general and administrative expenses, respectively, for employees, directors and non-employees for the nine months ended September 30, 2021 and 2020 is as follows:

	Nine Months Ended September 30,	
	2021	2020
Research and development	$ 1,051	$ 776
General and administrative	2,067	1,652
Total stock-based compensation expense	$ 3,118	$ 2,428

8. STOCKHOLDERS' EQUITY

Open Market Sale Agreement

On November 15, 2019, the Company entered into an Open Market Sale Agreement (the "Open Market Sale Agreement") with Jefferies LLC, as agent ("Jefferies"), and filed a related prospectus supplement, pursuant to which the Company may issue and sell shares of its common stock at the then current market prices having an aggregate offering price of up to $50,000 (the "Open Market Shares") from time to time through Jefferies (the "Open Market Offering").

Under the Open Market Sale Agreement, Jefferies may sell the Open Market Shares by methods deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Exchange Act of 1934, as amended. The Company may sell the Open Market Shares in amounts and at times to be determined by the Company from time to time subject to the terms and conditions of the Open Market Sale Agreement, but it has no obligation to sell any of the Open Market Shares in the Open Market Offering.

The Company or Jefferies may suspend or terminate the offering of Open Market Shares upon notice to the other party and subject to other conditions. The Company has agreed to pay Jefferies commissions for its services in acting as agent in the sale of the Open Market Shares in the amount of up to 3.0% of gross proceeds from the sale of the Open Market Shares pursuant to the Open Market Sale Agreement. The Company has also agreed to provide Jefferies with customary indemnification and contribution rights.

During the nine months ended September 30, 2021, the Company issued 922,077 Open Market Shares at a weighted-average price of $5.70 per share, resulting in net proceeds to the Company of $5,098. During the nine months ended September 30, 2020, the Company issued 436,477 shares of its common stock at a weighted-average price of $7.56 per share, resulting in net proceeds to the Company of $3,227. At September 30, 2021, the Company had $41,253 in aggregate gross offering amount available under the Open Market Sale Agreement.

9. REVENUE

Service Revenue

Takeda Agreement

In January 2020, the Company entered into a Research Collaboration and Option Agreement with Takeda ("Takeda Agreement"), which is accounted for within the scope of Accounting Standards Codification ("ASC") Topic 606, *Revenue from Contracts with Customers* ("ASC 606"). For further details on the terms and accounting treatment consideration for the Takeda Agreement, please refer to Note 10, *"Revenue,"* to the Company's consolidated financial statements contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2020.

During the three and nine months ended September 30, 2021, the Company recognized $43 and $556, respectively, in service revenue under the Takeda Agreement. During the three and nine months ended September 30, 2020, the Company recognized $926 and $2,912, respectively, in service revenue under the Takeda Agreement. As of September 30, 2021, there was $119 in accounts receivable on the consolidated balance sheet related to the Takeda Agreement.

Daiichi Sankyo Agreement

In April 2021, the Company entered into a Research Collaboration and Exclusive Option Agreement (the "Daiichi Agreement") with Daiichi for the development of gene therapy candidates for two indications based on the GeneRide platform (each, a "Daiichi Candidate"). Under the terms of the Daiichi Agreement, Daiichi will fund all research and development activities related to the development of the Daiichi Candidates under a mutually agreed research plan (the "Daiichi Research Plan"). The Daiichi Agreement also provides Daiichi with an exclusive, non-binding option for each Daiichi Candidate to negotiate in good faith for a certain period of time to enter into a license agreement with respect to each such Daiichi Candidate (the "Daiichi License Options").

The Company assessed the Daiichi Agreement in accordance with ASC 606 and concluded that it represents a contract with a customer and is within the scope of ASC 606. The Company concluded that its conduct of research services under the Daiichi Research Plan, which includes a research data package, participation in various joint oversight committees, a research license and a materials transfer, represents a single combined performance obligation. The Company determined the transaction price totaled $2,000, which included an upfront payment of $1,000 and an additional $1,000 prepayment of the first-year research and development fees. The entire transaction price will be allocated to the single combined performance obligation, which is transferred over the expected term of the conduct of the research services. Terms related to exclusive licenses negotiated after the exercise of the Daiichi License Options will be part of a separate contract and reflect applicable standalone selling prices. As such, the Company concluded the Daiichi License Options are not considered to be a material right.

The upfront payment of $2,000 was recorded as deferred revenue and is being recognized as revenue over time in conjunction with the Company's conduct of research services as the research services are the primary component of the combined performance obligation. Revenue associated with the upfront payment and ongoing research services will be recognized using an input-based measurement of actual costs incurred as a percentage of the estimated total costs expected to be incurred over the expected term of conduct of the research services. The Company believes this input-based method to recognize revenue best reflects the transfer of value to Daiichi. During the three and nine months ended September 30, 2021, the Company recognized $392 and $572, respectively, as service revenue under the Daiichi Agreement. As of September 30, 2021, there was $1,428 in deferred revenue related to the Daiichi Agreement, of which $1,130 was classified as current deferred revenue.

Collaboration Revenue

CANbridge Agreement

In April 2021, the Company entered into an Exclusive Research Collaboration, License and Option Agreement (the "CANbridge Agreement") with CANbridge.

Under the terms of the CANbridge Agreement, the Company granted CANbridge (a) an exclusive worldwide license to certain of the Company's intellectual property rights, including those relating to sL65, the first capsid produced from the sAAVy platform, to develop, manufacture and commercialize gene therapy candidates for the treatment of Fabry and Pompe diseases (the "Fabry and Pompe License"), (b) an option to obtain an exclusive worldwide license to certain of the Company's intellectual property rights, including those relating to sL65, to develop and commercialize gene therapy candidates for the treatment of two additional indications (the "Candidate Option") and (c) an exclusive option to obtain an exclusive license to develop and commercialize LB-001 for the treatment of MMA (the "LB-001 Option") in China, Taiwan, Hong Kong and Macau ("Greater China"). Pursuant to the CANbridge Agreement, LogicBio and CANbridge will collaborate to develop the gene therapy candidates referenced in (a) above for the treatment of Fabry and Pompe diseases plus, upon CANbridge's exercise of the applicable option, two additional indications under a

mutually agreed research plan. CANbridge agreed to provide funding for LogicBio's activities under the research plan in accordance with a mutually agreed research budget.

Under the CANbridge Agreement, the Company received an upfront, non-refundable and non-creditable payment of $10,000 from CANbridge. In addition, CANbridge is obligated to reimburse the Company for research and development costs incurred by the Company for activities related to the development of the gene therapy candidates for two indications, Pompe disease and Fabry disease, under a mutually agreed upon research plan (the "CANbridge Research Plan").

The Company is eligible to receive up to $542,000 in aggregate from CANbridge contingent on the achievement of specified clinical, regulatory and sales milestones relating to the named gene therapy candidates for Fabry disease and Pompe diseases, the additional indications for which CANbridge exercises the Candidate Option, and the payment of any option exercise fees. The Company is also eligible to receive up to $49,000 in aggregate clinical, regulatory and sales milestones for LB-001, subject to the exercise of the LB-001 Option, and the payment of the LB-001 Option fee. CANbridge is obligated to pay to the Company royalties based on an escalating tiered, mid- to high-single digit percentage of the annual worldwide net sales for each non-LB-001 indication pursued. In addition, CANbridge will pay to the Company royalties based on an escalating tiered, high-single digit to mid-double digit percentage of the annual Greater China net sales for LB-001 for the treatment of MMA, subject to the exercise of the LB-001 Option.

The Company applied ASC Topic 808, *Collaborative Arrangements* ("ASC 808") and determined that the CANbridge Agreement is within the scope of ASC 808. Furthermore, the Company determined that certain aspects of the CANbridge Agreement represented a vendor-customer relationship as CANbridge represents a customer for certain activities. As such, the Company applied the relevant guidance from ASC 606 to evaluate the appropriate accounting for the vendor-customer aspects of the CANbridge Agreement. In accordance with ASC 606, the Company identified its performance obligation as a grant of a license to CANbridge for certain of its intellectual property rights, including those relating to sL65, and its conduct of research services under the CANbridge Research Plan, which includes participation in various joint oversight committees and a technology transfer. The Company determined that its grant of a license to CANbridge to certain of its intellectual property subject to certain conditions was not distinct as it does not have stand-alone value to CANbridge apart from the services to be performed by the Company pursuant to the CANbridge Agreement. A third party would not be able to provide research and development services due to the specific nature of the intellectual property and knowledge required to perform the services, and CANbridge could not benefit from the license without the corresponding services. The Company also concluded that the LB-001 Option and Candidate Options were not provided to CANbridge at a significant discount. The terms of the options, including the upfront exercise fee and applicable milestone payments, reflected applicable standalone selling prices at the time of the CANbridge Agreement. As such, the Company concluded that none of the options was considered to be material rights and, as such, were not performance obligations.

Accordingly, the Company determined that its grant of a license to CANbridge and its conduct of research and development services under the research plan should be accounted for as one combined performance obligation, and that the combined performance obligation is transferred over the expected term of the conduct of the research and development services.

In accordance with ASC 606, the Company determined that the initial transaction price under the CANbridge agreement was $10,878, consisting of the upfront, non-refundable and non-creditable payment of $10,000 and an upfront payment of estimated quarterly research costs $878. The upfront payment of $10,878 was initially recorded as deferred revenue and, along with payments related to the Company's conduct of research services under the research plan, will be recognized as revenue using an input-based measurement of actual costs incurred as a percentage of the estimated total costs expected to be incurred over the expected term of conduct of the research services. The Company believes this input-based method to recognize revenue best reflects the transfer of value to CANbridge. The Company recorded the initial $878 prepayment of the quarterly research and development fees as deferred revenue, and such fees will be recognized as revenue as the research services are delivered.

The Company also assessed the effects of variable elements including the likelihood of receiving (i) various clinical, regulatory and commercial milestone payments, and (ii) royalties on net sales of any product candidate. Based on its assessment, the Company concluded that, based on the likelihood of these uncertain events occurring, there was not a significant variable element included in the transaction price. Accordingly, the Company has not assigned a transaction price to these variable elements given the substantial uncertainty related to their achievement and has not assigned a transaction price to any CANbridge milestone or royalties.

The Company recognized revenue of $1,685 and $2,255 under the CANbridge Agreement for the three and nine months ended September 30, 2021, respectively. As of September 30, 2021, aggregate deferred revenue related to the CANbridge Agreement was $9,305 of which $3,707 was classified as current. Both the current and non-current deferred revenue amounts will be recognized during the expected term of the conduct of research and development services. As a direct result of the Company's entry into the CANbridge Agreement, the Company incurred $775 in sublicense fees to certain of its existing licensors which was expensed to research and development expense during the quarter ended June 30, 2021.

10. INCOME TAXES

For the nine months ended September 30, 2021 and the year ended December 31, 2020, the Company maintained a full valuation allowance on federal and state deferred tax assets since management does not forecast the Company to be in a taxable position in the near future.

11. LOSS PER SHARE

Basic loss per share is computed by dividing net loss by the weighted-average shares of common stock outstanding, without consideration to common stock equivalents:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Numerator:				
Net loss	$ (10,185)	$ (8,039)	$ (30,966)	$ (25,721)
Denominator:				
Weighted-average common stock outstanding	32,443,960	23,599,052	32,181,912	23,367,804
Net loss per share — basic and diluted	$ (0.31)	$ (0.34)	$ (0.96)	$ (1.10)

The Company's potentially dilutive shares, which include any outstanding stock options, warrants and unvested restricted stock (which includes unvested restricted stock units and unvested restricted common stock), are considered to be common stock equivalents and are only included in the calculation of diluted net loss when their effect is dilutive.

The Company excluded the following potential common stock equivalents from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect for the three and nine months ended September 30, 2021 and 2020.

	September 30, 2021	September 30, 2020
Unvested restricted common stock	11,153	51,300
Unvested restricted stock units	5,939	120,437
Options to purchase common stock	4,508,640	2,835,149
Term A Loan warrants	15,686	15,686

12. LEASES

The Company has historically entered into lease arrangements for its facilities and certain equipment. As of September 30, 2021, the Company had one operating lease with required future minimum payments related to its headquarters in Lexington, MA.

In November 2019, the Company entered into a lease agreement for office, laboratory and vivarium space located at 65 Hayden Avenue, Lexington, Massachusetts ("65 Hayden Ave Lease") to replace the Company's prior headquarters in Cambridge, Massachusetts. Under the terms of the 65 Hayden Ave Lease, the Company leases approximately 23,901 square feet of space and is obligated to pay an initial annual base rent of approximately $1,494, which is subject to scheduled annual increases, plus certain operating expenses and taxes. The Company took possession of the space on April 1, 2020 ("Lease Commencement Date") and the lease will continue through July 1, 2025 ("Lease Termination Date"). The Company has an option to extend the lease for a single additional term of 5 years. Upon execution of the 65 Hayden Ave Lease, the Company executed a $622 cash-collateralized letter of credit. Lease payments are due in monthly installments through the Lease Termination Date.

At the Lease Commencement Date, the Company performed a lease assessment under the guidance prescribed in ASC Topic 842, *Leases* ("ASC 842"), and concluded that the 65 Hayden Ave Lease was an operating lease. As such, the Company recorded an operating lease right-of-use asset and corresponding operating lease liability on the consolidated balance sheets of $6,428 which reflected the net present value of future payments under the lease. The discount rate used to calculate the net present value of future payments was the Company's incremental borrowing rate at the Lease Commencement Date, which was 7.6%.

The following table contains a summary of the lease costs recognized under ASC 842 and other information pertaining to the Company's operating leases for the three and nine months ended September 30, 2021 and 2020:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Operating leases				
Lease cost				
Operating lease cost	$ 378	$ 465	$ 1,134	$ 1,254
Variable lease cost	208	190	639	474
Total lease cost	$ 586	$ 655	$ 1,773	$ 1,728
Other year-to-date lease information				
Operating cash flows used for operating leases			$ 385	$ 929
Operating lease liabilities arising from obtaining right-of-use assets			$ —	$ 6,428

The following table contains a summary of the lease liabilities recognized on the Company's condensed consolidated balance sheets as of September 30, 2021 and December 31, 2020:

	As of September 30, 2021	As of December 31, 2020
Other operating lease information		
Operating lease liabilities — short-term	$ 1,193	$ 1,094
Operating lease liabilities — long-term	$ 4,046	$ 4,952
Weighted-average remaining lease term	3.8 years	4.5 years
Weighted-average discount rate	7.60%	7.60%

The variable lease costs for the three and nine months ended September 30, 2021 and 2020 include common area maintenance and other operating charges. As the Company's leases do not provide an implicit interest rate, the Company utilized its incremental borrowing interest rate based on what it would normally pay to borrow on a collateralized basis over a similar term for an amount equal to the lease payments at the commencement date in determining the present value of lease payments.

Future minimum lease payments under the Company's operating lease as of September 30, 2021 and December 31, 2020, were as follows:

	As of September 30, 2021	As of December 31, 2020
Maturity of lease liabilities		
2021	$ 385	$ 1,516
2022	1,562	1,562
2023	1,609	1,609
2024	1,656	1,656
2025	841	841
Thereafter	—	—
Total lease payments	6,053	7,184
Less: imputed interest	(814)	(1,138)
Total operating lease liabilities	$ 5,239	$ 6,046

13. RELATED PARTIES

The Company is party to a consulting service agreement with Mark Kay, who is a co-founder and a member of the Board. Under the terms of this agreement, the Company has agreed to pay an annual fee of $68 for research and development consulting services. For each of the three and nine-month periods ended September 30, 2021 and 2020, the Company recorded research and development expense of $17 and $51, respectively, related to consulting services received from Mark Kay. In addition, as a result of his participation on the Scientific Advisory Board in June 2021, Mark Kay earned $5 and received a non-qualified stock option to

purchase 5,000 shares of the Company's common stock with a fair value of $13, which will be expensed over a two-year vesting period. Expenses related to Mark Kay's participation on the Scientific Advisory Board are recorded in research and development expense.

14. COMMITMENTS AND CONTINGENCIES

Litigation and Related Matters

From time to time, the Company may become subject to legal proceedings and claims which arise in the ordinary course of its business. Consistent with ASC 450, *Contingencies*, the Company's policy is to record a liability if a loss in a significant legal dispute is considered probable and an amount can be reasonably estimated. The Company provides disclosure when a loss in excess of any reserve is reasonably possible, and if estimable, the Company discloses the potential loss or range of possible loss. Significant judgment is required to assess the likelihood of various potential outcomes and the quantification of loss in those scenarios. The Company's estimates change as litigation progresses and new information comes to light. Changes in Company estimates could have a material impact on the Company's results and financial position. As of September 30, 2021, the Company did not have any significant legal disputes that require a loss liability to be recorded. The Company continually monitors the need for a loss liability for litigation and related matters.

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 condensed consolidated financial statements and the accompanying notes included in this Quarterly Report on Form 10-Q and the audited consolidated financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2020, which was filed with the U.S. Securities and Exchange Commission, or SEC, on March 15, 2021, or the 2020 10-K. This discussion and analysis contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth under "Risk Factors" in Part I, Item 1A. of the 2020 10-K, as may be amended or updated in subsequent filings with the SEC, our actual results may differ materially from those anticipated in these forward-looking statements.

Overview

We are a clinical-stage genetic medicine company pioneering gene editing and gene delivery platforms to address rare and serious diseases from infancy through adulthood. Our genome editing platform, GeneRide™, is a new approach to precise gene insertion harnessing a cell's natural deoxyribonucleic acid, or DNA, repair process potentially leading to durable therapeutic protein expression levels. Our gene delivery platform, sAAVy™, is an adeno-associated virus, or AAV, capsid engineering platform designed to optimize gene delivery for treatments in a broad range of indications and tissues.

Our lead product candidate, LB-001, is a single-administration, genome editing therapy developed using our GeneRide technology, currently in Phase 1/2 development for the treatment of methylmalonic acidemia, or MMA, in pediatric patients. MMA is a rare and life-threatening genetic disorder affecting approximately 1 in 50,000 newborns in the United States that often results in developmental delays and other long-term complications and a high rate of hospitalizations. In April 2021, we granted CANbridge Care Pharma Hong Kong Limited, or CANbridge, an exclusive option to obtain an exclusive license to develop and commercialize LB-001 for the treatment of MMA in China, Taiwan, Hong Kong and Macau.

Also based on our GeneRide technology, we completed the first phase of preclinical development of our product candidate, LB-301, for the treatment of Crigler-Najjar syndrome, a rare monogenic pediatric disease affecting approximately 1 in 1,000,000 newborns globally, in collaboration with Takeda Pharmaceutical Company Limited, or Takeda. In addition, we are developing treatments based on our GeneRide technology for two indications in collaboration with Daiichi Sankyo Company, or Daiichi. In addition, we demonstrated proof of concept of our GeneRide platform in hemophilia B and alpha-1-antitrypsin deficiency, or A1ATD, animal disease models, and more recently, in hereditary tyrosinemia type 1, or HT1, and Wilson disease.

Based on our sAAVy technology, we are developing gene therapy candidates utilizing, among other things, AAV sL65, the first capsid produced from sAAVy, for the treatment of Fabry and Pompe diseases in collaboration with CANbridge. We also granted CANbridge an option to obtain an exclusive worldwide license to certain of our intellectual property rights, including those relating to sL65, to develop and commercialize gene therapy candidates for the treatment of two additional indications.

We expect to select future product candidates from diseases addressed by targeting the liver initially, and later by targeting other tissues such as the central nervous system, or CNS, muscle, or other tissues. We plan to select one new development candidate from our preclinical portfolio by the end of 2021 and commence Investigational New Drug Application-enabling studies utilizing our modular approach and leveraging learnings from our lead programs.

LB-001 for the Treatment of Methylmalonic Acidemia (MMA) in Pediatric Patients

We are evaluating the safety, tolerability and preliminary efficacy of LB-001 in our Phase 1/2 SUNRISE clinical trial in pediatric patients with MMA. The SUNRISE trial is designed to enroll up to eight patients with ages ranging from six months to twelve years and evaluate a single administration of LB-001 at two dose levels (5×10^{13} vg/kg and 1×10^{14} vg/kg). On June 2, 2021, we announced that the first patient was dosed in the SUNRISE trial. In accordance with the FDA-cleared protocol, we initially enrolled patients in the three- to twelve-year-old age group at the lower dose.

On October 18, 2021, we announced early results from the SUNRISE trial. The early results showed measurable levels of albumin-2A, a technology-related biomarker indicating site-specific gene insertion and protein expression. Detection of albumin-2A is an indication that we have achieved the first ever *in vivo* genome editing in children. On October 18, 2021, we also announced that, following an evaluation of the safety data from the first two patients enrolled in the SUNRISE trial, the independent Data Safety Monitoring Board, or the DSMB, overseeing the SUNRISE trial recommended continuation of the trial without modification. Albumin-2A detection together with the DSMB continuation recommendation enabled us to begin enrolling two patients in the higher dose cohort (with ages ranging three to twelve years old), and two patients in the lower age (six months to two years old) cohort at the

lower dose. In late October 2021, the third patient dosed in the SUNRISE trial, who received 5 x 10^{13} vg/kg of LB-001 and was in the six-month to two-year-old age group, experienced a serious adverse event, or SAE, deemed possibly related to study drug. The SAE was noted during a scheduled visit that took place two weeks after dosing. This SAE is being ultimately categorized as a case of thrombotic microangiopathy, or TMA, which has been previously reported in association with other adeno-associated virus genetic therapies, and classically presents as a syndrome of hemolytic anemia, thrombocytopenia, and acute kidney injury. While the SAE experienced by this patient was characterized by hemolytic anemia and acute kidney injury, thrombocytopenia was not present. The patient was hospitalized, monitored and responded well to intravenous fluids and parenteral nutrition. The patient is currently asymptomatic. Key laboratory parameters that were abnormal at the time of the visit have either returned to normal or improved, and following a hospitalization of less than a week, the patient is continuing outpatient assessments in accordance with the protocol. We reported the SAE to the DSMB along with a proposed response plan, which was endorsed by the DSMB. The plan includes proposed protocol amendments to increase patient monitoring. In accordance with our regulatory obligations, we also reported the SAE to the FDA. We plan to continue to enroll the study in accordance with the protocol and additional safety measures we have communicated to the sites.

In accordance with the protocol, following the dosing of two patients in the lower age cohort and two patients from the older age cohort with the higher dose, the SUNRISE trial could progress to dosing additional patients in the younger age group at the higher dose (cohort 2, younger age group, n=2), subject to a review of safety data and/or albumin-2A detection, as applicable. The SUNRISE trial includes a six-week staggering interval between the dosing of each patient with the exception that age de-escalation and dose escalation can occur simultaneously. Patients participate in a pre-dosing observational period and are administered a prophylactic steroid regimen.

The primary endpoint of the SUNRISE trial is to assess the safety and tolerability of LB-001 at 52 weeks after a single infusion. Additional endpoints include changes in disease-related biomarkers, including serum methylmalonic acid, clinical outcomes such as growth and healthcare utilization, and the pharmacodynamic marker albumin-2A. We expect seven centers in the United States and one center in Saudi Arabia to participate in the SUNRISE trial. Based on current projections for enrollment, we expect to announce interim data by the end of 2021.

In addition to the Phase 1/2 SUNRISE trial, we have also completed a retrospective natural history study designed to evaluate disease progression in pediatric patients with MMA. These data helped to provide us with a better understanding of the natural progression of the disease, the impact of a liver transplant on the outcomes of MMA patients and potential endpoints such as the relevance of methylmalonic acid levels on clinical outcomes, with the goal of informing our future clinical development in MMA and our discussions with regulatory agencies as we look toward advancing our MMA program. We presented preliminary findings from our retrospective natural history study at the American College of Medical Genetics in April 2021.

In July 2019, the U.S. Food and Drug Administration, or FDA, granted rare pediatric disease designation for LB-001 for the treatment of MMA, and in April 2019, the FDA granted orphan drug designation for LB-001 for the treatment of MMA. In November 2020, the FDA granted fast track designation for LB-001 for the treatment of MMA, and in June 2021, the European Commission granted orphan drug designation to LB-001 for the treatment of MMA.

Our GeneRide Platform

Our genome editing platform, GeneRide, is a new approach to precise gene insertion harnessing a cell's natural DNA repair process potentially leading to durable therapeutic protein expression levels. GeneRide is designed to support the development of a new generation of genetic medicines designed to insert a corrective gene in the human genome with the goal of avoiding certain risks associated with other methods of gene therapy and gene editing. The therapies developed based on our GeneRide platform are designed to use an engineered viral vector to deliver a corrective gene, known as a transgene, to the nuclei of a patient’s cells utilizing a one-time infusion.

We believe that GeneRide has the potential to enable us to target diseases that cannot be treated by current genetic medicines, including early onset genetic childhood diseases where early intervention is critical in order to prevent progressive irreversible tissue damage that leads to long-term complications.

In addition, preclinical studies have shown that some specific product candidates targeting the liver based on the GeneRide platform have exhibited “selective advantage,” where modified cells take over the non-modified cells as the liver continues to regenerate, which allowed expression of therapeutic levels of the missing protein.

At the European Society of Gene & Cell Therapy, or ESGCT, conference in October 2021, we presented preclinical data that validates previous research in MMA and highlights selective advantage in two additional indications characterized by intrinsic liver damage,

HT1, and Wilson disease. In the HT1 mice models with acute liver damage, the data showed that GeneRide-corrected hepatocytes repopulated the entire liver within weeks post-administration, replacing the diseased hepatocytes with corrected hepatocytes. These mice models demonstrated restored normal body growth, liver function, and undetectable succinyl acetone levels, one of the toxic metabolites that accumulates in patients with HT1. In a Wilson disease mouse model, GeneRide-corrected hepatocytes partially repopulated the liver within months, and the treated mice showed improvements in liver function, hepatomegaly, and urinary copper excretion.

Our sAAVy Platform

We are also developing sAAVy, a next generation AAV capsid platform, for use in gene editing and gene therapy. At the American Society of Gene & Cell Therapy, or ASGCT, 2020 Annual Meeting in May 2020, we presented data showing that the capsids delivered highly efficient functional transduction of human hepatocytes in a humanized mouse model. Based on these data, we believe the top-tier capsid candidates from this effort demonstrated the potential to achieve significant improvements over benchmark AAVs that are currently in clinical development. We are developing these highly potent vectors for use in our internal development candidates and collaborations. We announced data generated from translational animal models using these capsids at the ASGCT 2021 Annual Meeting in May 2021. In addition, in January 2021, we announced the extension of our collaboration with Children's Medical Research Institute, or CMRI, to continue to develop next-generation capsids for gene therapy and gene editing applications in the liver as well as two additional tissues.

Operating Overview

Since our inception in 2014, we have devoted the majority of our efforts to research and development, including our preclinical and clinical development activities and our manufacturing and process development activities, raising capital, and providing general and administrative support for these operations. We do not have any products approved for sale and our only revenue recognized to date has been revenue related to upfront payments and research cost reimbursement under our strategic agreements with CANbridge, Daiichi and Takeda. Through September 30, 2021, we have raised approximately $126.0 million through underwritten public offerings and at-the-market sales of our common stock and $33.1 million in net proceeds from the sale of preferred stock prior to our initial public offering. In July 2019, we entered into a Loan and Security Agreement for term loans with Oxford Finance LLC and Horizon Technology Finance Corporation, or the Loan Agreement, under which term loans in an aggregate principal amount of $20.0 million were made available to us in two tranches, subject to certain terms and conditions. As of September 30, 2021, we had drawn down the $10.0 million first tranche. In the second quarter of 2021, we met the conditions to initiate drawdown of the $10.0 million second tranche but did not exercise our right to do so and the option to draw down the second tranche of the Term Loans expired. 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 our product candidate and any future product candidates. Our net loss was $31.0 million for the nine months ended September 30, 2021. As of September 30, 2021, we had an accumulated deficit of $131.0 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future in connection with our ongoing activities. While we intend to continue to evaluate ways to enhance our liquidity and capital position, our efforts will largely depend on future developments that are highly uncertain and cannot be predicted with confidence at this time.

Impact of COVID-19

We are closely monitoring the COVID-19 pandemic in order to promote the safety of our personnel and to continue advancing our research and development activities. We are following federal, state and local requirements and guidelines with respect to the COVID-19 pandemic, and have allowed our employees to return to working on-premises in accordance with those requirements and guidelines.

The COVID-19 pandemic did not have a material impact on our results of operations, cash flow and financial position as of and for the nine months ended September 30, 2021. However, we are aware that certain of our third-party vendors are being affected by import/export and other restrictions due to the COVID-19 pandemic, which are currently having an impact on certain of our research, development and manufacturing activities. Further, the pandemic could also potentially affect the business of the FDA, the EMA or other governmental authorities, which could result in delays in meetings, reviews, inspections and approvals relating to LB-001. Any decision by the FDA, EMA or other governmental authorities to delay meeting with us or scheduling inspections in light of COVID-19 could have a material adverse effect on our clinical trials, which could increase our operating expenses and have a material adverse effect on our financial results, including the timing and amount of future regulatory milestones we could receive from our partners.

We cannot predict the impact of the progression of the COVID-19 pandemic on future results due to a variety of factors, including the health of our and their employees, our ability to maintain operations, the ability of our third-party vendors, suppliers and collaborators

to continue operations, any further government and/or public actions taken in response to the pandemic and ultimately the length of the pandemic.

We plan to continue to closely monitor the COVID-19 pandemic in order to ensure the safety of our personnel and to continue advancing our research and development activities.

Components of Results of Operations

Revenue

Our only revenue recognized to date has been revenue related to upfront payments and research cost reimbursement under our agreements with CANbridge, Daiichi and Takeda. We have not generated any revenue from product sales and do not expect to generate any revenue from product sales for the foreseeable future.

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 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;
- 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 and related 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. 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 increase in the future as we continue to conduct the clinical program for our product candidate, LB-001, and as we increase our research and development headcount to continue to discover and develop additional product candidates. If any of our product candidates enter into later stages of clinical development, we expect that 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.

Other Income (Expense), Net

Interest income consists primarily of interest on our cash and cash equivalents and investments. Interest expense consists of interest expense related to the aggregate $10.0 million principal amount of the Term A Loan borrowing under the loan agreement in July 2019. A portion of the interest expense on the Term A Loan is non-cash expense relating to the accretion of the debt discount, amortization of issuance costs and accretion of the final payment fee. During each of the three and nine months ended September 30, 2021 and 2020, we recorded $0.3 million and $0.8 million, respectively, in interest expense, of which $0.2 million and $0.7 million, respectively, related to cash interest paid and the remainder to the accretion of the debt discount and amortization of issuance costs.

Results of Operations

Comparison of the Three Months Ended September 30, 2021 and 2020

The following table summarizes our results of operations for the three months ended September 30, 2021 and 2020:

	Three Months Ended September 30,	
	2021	2020
	(in thousands)	
REVENUE		
Collaboration and service revenue	$ 2,120	$ 926
Total revenue	2,120	926
OPERATING EXPENSES		
Research and development	7,806	5,492
General and administrative	4,257	3,200
Total operating expenses	12,063	8,692
LOSS FROM OPERATIONS	(9,943)	(7,766)
OTHER (EXPENSE) INCOME:		
Other expense, net	(270)	(273)
Loss before income taxes	(10,213)	(8,039)
Income tax provision	28	—
Net loss	$ (10,185)	$ (8,039)

Revenue

Our revenue for the three months ended September 30, 2021 consisted of $2.1 million, relating to our agreements with CANbridge, Daiichi and Takeda. Our revenue for the three months ended September 30, 2020 consisted of $0.9 million in revenue under the Takeda agreement. The increase in revenue during the three months ended September 30, 2021 compared to the corresponding period in 2020 was related to revenue recognized under the April 2021 CANbridge and Daiichi agreements, which was partially offset by a decrease in revenue under the Takeda Agreement due to winding down activities. We expect our collaboration and service revenue to increase in the fourth quarter of 2021, as compared to the third quarter of 2021, as we continue to conduct activities under the CANbridge and Daiichi agreements.

Research and Development Expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2021 and 2020:

	Three Months Ended September 30,		
	2021	2020	Increase
	(in thousands)		
LB-001 external development and manufacturing costs	$ 2,207	$ 1,714	$ 493
Personnel-related costs	2,778	1,536	1,242
Lab supplies	1,068	642	426
Other research and development costs	1,753	1,600	153
Total research and development expenses	$ 7,806	$ 5,492	$ 2,314

Research and development expenses for the three months ended September 30, 2021 were $7.8 million, compared to $5.5 million for the three months ended September 30, 2020. The increase of approximately $2.3 million was primarily due to increases of $1.2 million in personnel-related costs related to an increase in headcount associated with the progress of both our partnered and internal programs and a corresponding increase of $0.4 million in lab supplies. In addition, LB-001 external development and manufacturing costs increased $0.5 million mainly driven by an increase in activities supporting the LB-001 development program. We expect that our research and development expenses will increase during the fourth quarter as we continue to advance our pipeline both internally and with collaborators as well as continue to advance our LB-001 clinical program.

General and Administrative Expenses

General and administrative expenses were $4.3 million for the three months ended September 30, 2021, compared to $3.2 million for the three months ended September 30, 2020. The increase of approximately $1.1 million was primarily driven by an increase of $0.4 million in personnel expenses as we increased our headcount to support our continued research and development activities as well as build our corporate and administrative functions, as well as an increase of $0.4 million in fees relating to professional services due to an increase in general corporate activities. We expect that our general and administrative expenses will remain relatively consistent during the fourth quarter of 2021 as compared to the third quarter of 2021.

Other Expense, Net

Other expense, net was $0.3 million for each of the three-month periods ended September 30, 2021 and 2020. Other expense, net remained consistent due to similar interest rates on our capital balances and principal amount due on the term loan balance during the periods.

Comparison of the Nine Months Ended September 30, 2021 and 2020

The following table summarizes our results of operations for the nine months ended September 30, 2021 and 2020:

	Nine Months Ended September 30,	
	2021	2020
	(in thousands)	
REVENUE		
Collaboration and service revenue	$ 3,383	2,912
Total revenue	3,383	2,912
OPERATING EXPENSES		
Research and development	21,482	18,560
General and administrative	12,081	9,421
Total operating expenses	33,563	27,981
LOSS FROM OPERATIONS	(30,180)	(25,069)
OTHER (EXPENSE) INCOME:		
Other expense, net	(814)	(652)
Loss before income taxes	(30,994)	(25,721)
Income tax provision	28	—
Net loss	$ (30,966)	$ (25,721)

Revenue

Our revenue for the nine months ended September 30, 2021 consisted of $3.4 million in revenue related to the Takeda, Daiichi and CANbridge agreements. Our revenue for the nine months ended September 30, 2020 consisted of $2.9 million in revenue under the Takeda agreement. The increase in revenue for the nine months ended September 30, 2021 compared to the corresponding period in 2020 was related to revenue recognized under the April 2021 CANbridge and Daiichi agreements which was partially offset by a decrease in revenue under the Takeda Agreement due to winding down activities.

Research and Development Expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2021 and 2020:

	Nine Months Ended September 30,		
	2021	2020	(Decrease)/Increase
	(in thousands)		
LB-001 external development and manufacturing costs	$ 5,905	$ 7,601	(1,696)
Personnel-related costs	7,270	4,700	2,570
Lab supplies	2,662	2,188	474
Other research and development costs	5,645	4,071	1,574
Total research and development expenses	$ 21,482	$ 18,560	$ 2,922

Research and development expenses for the nine months ended September 30, 2021 were $21.5 million, compared to $18.6 million for the nine months ended September 30, 2020. The increase of approximately $2.9 million was primarily due to an increase of $2.6 million in personnel-related costs related to an increase in headcount associated with the progress of both our partnered and internal programs, with a corresponding increase of $0.5 million in lab supplies. During the nine months ended September 30, 2021 as compared to the nine months ended September 30, 2020, there was an increase of $1.6 million in other research and development costs, primarily driven by intellectual property licensing obligations due to certain of our licensors and increased collaboration costs. These increases were partially offset by a $1.7 million decrease in LB-001 external development and manufacturing costs, which primarily consisted of a $3.4 million decrease in contract manufacturing costs for LB-001 clinical supply offset by an increase of $1.8 million in expenses related to the clinical trials.

General and Administrative Expenses

General and administrative expenses were $12.1 million for the nine months ended September 30, 2021, compared to $9.4 million for the nine months ended September 30, 2020. The increase of approximately $2.7 million was primarily driven by an increase of $1.1

million in personnel expenses as we increased our headcount to support our continued research and development activities as well as build our corporate and administrative functions. There was also a $1.0 million increase in professional service fees due to an increase in corporate development and general corporate activities as well as $0.6 million increase in other general and administrative costs primarily related to an increase in corporate communications, insurance, and other general and administrative expenses.

Other Expense, Net

Other expense, net was $0.8 million for the nine months ended September 30, 2021 compared to other expense, net of $0.7 million for the nine months ended September 30, 2020. The increase in other expense, net was primarily related to a decrease in interest income due to lower interest rates beginning in March 2020.

Liquidity and Capital Resources

Overview

Since our inception and through September 30, 2021, we have not generated any sales revenue and have incurred significant losses and negative cash flows from our operations.

As of September 30, 2021, we had cash and cash equivalents of $59.6 million, which we believe will be able to fund our operating expenses and capital expenditure requirements into the fourth quarter of 2022. On a quarterly basis, we are required to conduct an accounting analysis under ASC 205-40, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, or ASC 205-40. The result of our ASC 205-40 analysis is that there is substantial doubt about our ability to continue as a going concern within one year of the date these financial statements are filed. While we intend to evaluate ways to enhance our liquidity and capital position, our efforts will largely depend on future developments that are highly uncertain and cannot be predicted with confidence at this time. Our plans to mitigate the conditions that raise substantial doubt include raising additional capital through equity or debt financings, payments from our collaborators, strategic transactions, or a combination of those approaches. These plans may also include the possible deferral of certain operating expenses unless and until additional capital is received. There can be no assurance that we will be successful in raising additional capital or that such capital, if available, will be on terms that are acceptable to us, or that we will be successful in deferring certain operating expenses.

Cash Flows

The following table summarizes our cash flows for the nine months ended September 30, 2021 and 2020:

	Nine Months Ended September 30,	
	2021	2020
	(in thousands)	
Net cash used in operating activities	$ (13,834)	$ (21,436)
Net cash (used in) provided by investing activities	(734)	17,157
Net cash provided by financing activities	4,074	3,311
Net decrease in cash, cash equivalents and restricted cash	$ (10,494)	$ (968)

Operating Activities

During the nine months ended September 30, 2021, net cash used in operating activities was $13.8 million, primarily related to our net loss adjusted for non-cash charges and changes in the components of working capital. The $7.6 million decrease in net cash used in operating activities during the nine months ended September 30, 2021, as compared to the nine months ended September 30, 2020, was primarily driven by a $10.7 million increase in deferred revenue related to the upfront consideration received under the CANbridge and Daiichi agreements and partially offset by an increase in cash operating expenses.

Investing Activities

During the nine months ended September 30, 2021, net cash used in investing activities was $0.7 million related to the purchases of property and equipment. During the nine months ended September 30, 2020, net cash provided by investing activities was $17.2 million primarily related to the proceeds from our short-term investments that matured during the period which were not reinvested and instead held as cash and cash equivalents.

Financing Activities

During the nine months ended September 30, 2021, net cash provided by financing activities was $4.1 million, primarily related to net proceeds from sales of our common stock under an Open Market Sale Agreement with Jefferies LLC as the sales agent, or the Open Market Sale Agreement, of $5.1 million which was partially offset by the repayment of principal on our term loans of $1.1 million. During the nine months ended September 30, 2020, net cash provided by financing activities was $3.3 million, primarily related to sales of common stock under the Jefferies LLC agreement of $3.2 million.

Funding Requirements

We expect to continue to incur a significant amount of expenses in connection with our ongoing activities for the foreseeable future. In particular, we will incur significant expenses related to the preclinical activities and clinical trials of our product candidates and any future product candidates.

We expect that our expenses will increase substantially if and as we:

- continue our research and preclinical development of any product candidates from our current or future research programs;
- continue to conduct our clinical program for LB-001 and initiate and conduct clinical trials for 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 completes 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 preclinical and clinical 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 and sAAVy platforms;
- hire additional technical, quality, regulatory, clinical, scientific and commercial personnel and 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 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 approval;
- lease and build new facilities, including offices and labs, to support organizational growth;
- comply with good practice quality guidelines and regulations, or GXP, including good laboratory practice, good clinical practice, or GCP, and current good manufacturing practice, or cGMP; establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval; and
- experience any delays or encounter issues with any of the above.

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 because of the numerous risks and uncertainties associated with the development of our lead product candidate, LB-001, and any other product candidates and programs we may develop and because the extent to which we may enter into collaborations with third parties for development of LB-001 and any other product candidates we may develop is unknown.

At September 30, 2021, we had $59.6 million of cash and cash equivalents on hand, which we currently expect will be sufficient to fund our operating expenses and capital expenditure requirements into the fourth quarter of 2022. We have based these estimates on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including:

- the scope, progress, timing, costs and results of drug discovery, preclinical development, laboratory testing, and clinical trials for our product candidates, including the ongoing development program for LB-001, which includes our Phase 1/2 SUNRISE clinical trial of LB-001 in MMA, and process development and manufacturing activities for LB-001;
- the outcome, timing and cost of following the advice of and complying with regulatory requirements and decisions made by the FDA, EMA and other regulatory authorities, including resolving any potential clinical holds that may be imposed on us;
- the impact of the COVID-19 pandemic on our ability to progress with our research, development, manufacturing and regulatory efforts, including our ability to advance and complete our Phase 1/2 SUNRISE clinical trial of LB-001;
- 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 and other research and development costs under future collaboration agreements, if any;
- the effect of competing technological and market developments;
- the cost and timing of completion of process development and manufacturing activities;
- the extent to which we engage in transactions, including collaboration, merger, acquisition and licensing transactions;
- 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;
- the initiation, progress, timing and results of our commercialization of LB-001 and any other product candidates, if approved, for commercial sale;
- our ability to repay outstanding debt; and
- our ability to attract, hire and retain qualified personnel.

A change in the outcome of any of the above variables that are applicable to the development of a product candidate could mean a significant change in the costs and timing associated with the research and 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. Any significant delays in our programs may also require us to reevaluate our corporate strategy, resulting in the expenditure of significant resources and time. We may never succeed in obtaining regulatory approval for our product candidates or any future product candidates.

Until such time, if ever, that we can generate product revenue sufficient to achieve profitability, we expect to finance our future cash needs through equity or debt financings, payments from our collaborators, strategic transactions, or a combination of those approaches. There is no assurance that we will be successful in obtaining any additional financing on terms acceptable to the Company, if at all. Additionally, the terms of any 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, reduce or eliminate some or all of our research and development programs, preclinical and clinical development programs or future commercialization efforts, or we may be unable to continue operations.

At-the-Market Sales of Common Stock

In November 2019, we entered into the Open Market Sale Agreement. Under the terms of the Open Market Sale Agreement and the related prospectus supplement we filed with the SEC in November 2019, we may sell shares of our common stock at the then current market prices, from time to time, having an aggregate value of up to $50.0 million through Jefferies LLC. We pay up to a 3% cash commission to Jefferies LLC on the proceeds from sales under the program. During the nine months ended September 30, 2021, we issued 922,077 shares of our common stock at a weighted-average price of $5.70 per share, resulting in net proceeds to us of $5.1 million. During the nine months ended September 30, 2020, we issued 436,477 shares of our common stock at a weighted-average price of $7.56, resulting in net proceeds to us of $3.2 million. At September 30, 2021, we had $41.3 million in aggregate gross offering amount available under the Open Market Sale Agreement and the related prospectus supplement.

Contractual Obligations and Commitments

We are a smaller reporting company, as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, for this reporting period and are not required to provide additional information on our contractual obligations and commitments pursuant to Item 303 of Regulation S-K.

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 2020 10-K.

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.

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 unaudited 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 a smaller reporting company, as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, for this reporting period and are not required to provide the information required under this item.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer), evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2021. The term

“disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act are recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the Company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2021, our Chief Executive Officer and our Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during the quarter ended September 30, 2021 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

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.

We face a variety of risks and uncertainties in our business. Additional risks and uncertainties not presently known to us or that we currently believe to be immaterial may also become important factors that affect our business. If any of the following risks occurs, our business, financial statements 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.

Investment in pharmaceutical product development and commercialization is highly speculative because it entails upfront capital expenditures and significant risk that a product candidate will fail to gain marketing approval or that an approved product will not be commercially viable. We have incurred net losses in each year since our inception, including net losses of $31.0 million for the nine months ended September 30, 2021. As of September 30, 2021, we had an accumulated deficit of $131.0 million. In addition, we have not commercialized any products and have never generated any revenue from product sales. Since our inception, we have devoted most of our resources to research and development, including our preclinical and clinical development activities as well as to building out our team and infrastructure. We expect to continue to incur significant additional operating losses for the foreseeable future as we seek to continue to enroll patients in SUNRISE and continue to advance LB-001, our lead product candidate, through clinical development, expand our research and development capabilities and activities, develop new product candidates and advance them through preclinical and clinical development, advance the development of our GeneRide and sAAVy technology platforms, conduct process development and manufacturing activities, conduct clinical trials, seek regulatory approval and, if we receive approval from the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or EMA, or other regulatory authorities, 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;
- continue to conduct our clinical program for LB-001 and initiate and conduct clinical trials for any other product candidates we identify and develop;
- seek to identify, assess, acquire and/or develop additional research programs and additional product candidates;
- engage in transactions, including strategic, merger, collaboration, acquisition and licensing transactions;
- seek regulatory approvals for any product candidates that successfully complete clinical trials; develop, optimize, scale and validate a manufacturing process and analytical methods for any product candidates we may develop;
- 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 and sAAVy technology platforms;
- hire additional scientific, clinical, technical, quality, regulatory, general and administrative, and commercial personnel and 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 our development programs 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;
- comply with good practice quality guidelines and regulations, or GXP, including good laboratory practice, good clinical practice, or GCP or cGMP;
- build out and validate clinical and commercial-scale cGMP manufacturing capabilities;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval; and
- experience any delays or encounter issues with any of the above.

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, along with our GeneRide and sAAVy platforms, will require additional preclinical and clinical development, 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.

Under our ASC 205-40 analysis, there is "substantial doubt" that we will have sufficient funds to satisfy our obligations through the next twelve months from the date of issuance of this Quarterly Report on Form 10-Q.

At September 30, 2021, we had $59.6 million of cash and cash equivalents on hand. On a quarterly basis, we are required to conduct an accounting analysis under ASC 205-40, Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern, or ASC 205-40. The result of our ASC 205-40 analysis is that there is "substantial doubt" that we will have sufficient funds to satisfy our obligations through the next twelve months from the date of issuance of this Quarterly Report on Form 10-Q. Our ability to continue as a going concern is dependent on our ability to obtain the necessary financing to meet our obligations and repay our abilities arising from the ordinary course of business operations when they become due. The outcome of these matters cannot be predicted with any certainty at this time. If we are unable to raise sufficient capital when needed, our business, financial condition and results of operations will be materially and adversely affected, and we will need to significantly modify our operational plans. Our conclusion, in accordance with ASC 205-40, that there is "substantial doubt" that we will have sufficient funds to satisfy our obligations through the next twelve months from the date of issuance of this Quarterly Report on Form 10-Q may materially adversely affect our business, prospects, financial condition, results of operations, share price and our ability to raise capital or to enter into agreements with third parties that may be beneficial to us.

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. For more information, see the risk factor titled "– Under our ASC 205-40 analysis, there is 'substantial doubt' that we will have sufficient funds to satisfy our obligations through the next twelve months from the date of issuance of this Quarterly Report on Form 10-Q." 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.

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 scope, progress, timing, costs and results of drug discovery, preclinical development, laboratory testing, and clinical trials for our product candidates, including the ongoing development program for LB-001, which includes our Phase 1/2 SUNRISE clinical trial of LB-001 in MMA, and process development and manufacturing activities for LB-001;
- the outcome, timing and cost of following the advice of and complying with regulatory requirements and decisions made by the FDA, EMA and other regulatory authorities, including resolving any potential clinical holds that may be imposed on us;
- the impact of the COVID-19 pandemic on our ability to progress with our research, development, manufacturing and regulatory efforts, including our ability to advance and complete our Phase 1/2 SUNRISE clinical trial of LB-001;
- 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 and other research and development costs under future collaboration agreements, if any;
- the effect of competing technological and market developments;
- the cost and timing of completion of process development and manufacturing activities;
- the extent to which we engage in transactions, including collaboration, merger, acquisition and licensing transactions;
- 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;
- the initiation, progress, timing and results of our commercialization of LB-001 and any other product candidates, if approved, for commercial sale;
- our ability to repay outstanding debt; and
- our ability to attract, hire and retain qualified personnel.

Identifying potential product candidates and conducting preclinical testing, process development, manufacturing activities 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 be required to delay, reduce or eliminate some or all of our research and development programs, preclinical and clinical development programs or future commercialization efforts, or we may be unable to continue operations. Any significant delays in our programs may also require us to reevaluate our corporate strategy, resulting in the expenditure of significant resources and time, or potentially resulting in us discontinuing our operations.

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 convertible debt securities or equity, including through our existing at-the-market equity facility, 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. For information about our current outstanding debt, see Note 6 in the accompanying notes to our unaudited condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the risk factor titled "The terms of our Loan and Security Agreement place restrictions on our operating and financial flexibility." 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 product sales 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 development of any product candidates we may identify;
- obtain sufficient financial and other resources to complete the LB-001 development program and the development programs of any other product candidate;
- obtain data from clinical programs of our product candidates that supports an acceptable risk-benefit profile of each such product candidate in the intended populations, including LB-001 in pediatric patients with MMA;
- develop safe and effective delivery mechanisms for our genome editing therapeutic product candidates;
- achieve desirable medicinal properties for the intended indications;
- seek and obtain regulatory approvals for any product candidate for which we complete clinical trials;
- launch and commercialize any product candidate for which we obtain regulatory approval by establishing a sales force, marketing and distribution infrastructure and/or 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 approval;
- develop, enhance, scale and validate a manufacturing process and analytical methods for any product candidates we may develop;
- 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 approval;
- compete with other therapies and treatment options;
- obtain market acceptance of any product candidates we may develop as viable treatment options;
- 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 and gene therapy we are subject to the following additional challenges and risks, 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, or the EU;
- the FDA's recommendation of a follow-up observation period of up to 15 years or longer for all patients who receive treatment using gene delivery , necessitating us to adopt such an observation period for any product candidate we may develop;
- gene delivery technologies are novel approaches and may present additional challenges and risks, including obtaining approval from regulatory authorities that have limited experience with the development of such technologies;
- reports may arise from preclinical or clinical testing of the gene delivery technologies of our competitors that may raise safety or efficacy concerns about our product candidates.

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

We intend to identify and develop product candidates based on our novel GeneRide and sAAVy technology platforms, 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 and only a few gene therapy products have been approved in the United States and Europe. In addition, there have only been a limited number of human clinical trials involving a gene editing and gene therapy product candidates, and our Phase 1/2 SUNRISE clinical trial is the first clinical trial of a product candidate utilizing our nuclease-free genome editing technology.

We have concentrated our research and development efforts on product candidates utilizing our GeneRide and sAAVy technology platforms. Our future success depends on the successful development of these novel therapeutic approaches. To date, no product that utilizes our GeneRide or sAAVy technology has been approved. There have been a limited number of clinical trials of gene editing and gene therapy technologies, however no genome editing product candidates have been approved and only a few gene therapy products have been approved in the United States and Europe. Our Phase 1/2 SUNRISE clinical trial is the first clinical trial of a product candidate that utilizes our technology. In addition, because our Phase 1/2 SUNRISE clinical trial of LB-001, our lead product candidate based on our technology is our first clinical trial of LB-001, we have not yet been able to fully assess safety in humans. In late October 2021, the third patient dosed in the SUNRISE trial, who received 5 x 10^{13} vg/kg of LB-001 and was in the six-month to two-year-old age group, experienced a serious adverse event, or SAE, deemed possibly related to study drug. For more information, see "LB-001 for the Treatment of Methylmalonic Acidemia (MMA) in Pediatric Patients" in "Management's Discussion and Analysis of Financial Condition and Results of Operations." Moreover, 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 non-human animal DNA differs from human DNA, results of tests of our product candidates in preclinical animal models for either safety or efficacy may not be predictive of results that may be observed in humans. Also, preclinical animal models may not exist for some of the diseases we expect to pursue. Our GeneRide genome editing and sAAVy technologies harness 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 predict whether any of our product candidates will be able to successfully and consistently integrate corrective DNA in or deliver gene transfer constructs to enough tissue cells or otherwise result in sufficient expression of the target protein 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 SAEs, such as the SAE referenced above, or toxicities. As a result of these factors, it is difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our GeneRide or sAAVy technology platforms, or any similar or competitive gene delivery 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 or sAAVy technology platforms 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 collaboration 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 gene delivery 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 gene delivery is novel, the regulatory requirements governing any gene delivery product candidates we develop are uncertain and subject to change. For example, the FDA issued several guidance documents regarding gene therapy in July 2018 and January 2020. The FDA also issued a guidance document in January 2021 addressing manufacturing considerations for licensed and investigational gene therapy products during the COVID-19 pandemic. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for global 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 genome editing, gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Genome editing and 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. Although the FDA decides whether individual genome editing or 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 to regulatory bodies in other jurisdictions, including Saudi Arabia, where our Phase 1/2 SUNRISE clinical trial is expected to be conducted, and 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.

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 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 and very few gene therapy products have 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 or gene therapy technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays or other impediments to our research 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 limited experience conducting clinical trials and no history 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 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 such as our GeneRide and sAAVy technology platforms, identifying and developing LB-001, undertaking preclinical studies, business planning and raising capital. Other than LB-001, which is in clinical development, all of our programs are still in the preclinical or research stage of development. The risk of failure in the biopharmaceutical industry for programs or product candidates at such stage of development is high. We have not yet demonstrated an ability to successfully complete any interventional clinical trials, including pivotal clinical trials, obtain marketing approval, manufacture a 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.

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 and development focus to a company capable of supporting commercial activities, if any, of our product candidates is approved. 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 and 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 Investigational New Drug Application, or 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. For example, our IND for LB-001 was placed on clinical hold by the FDA in February 2020 in order to evaluate certain clinical and preclinical aspects of our submission. While the clinical hold for LB-001 was lifted in August 2020, there can be no assurances that the FDA will not place this IND, or any IND relating to any other of our product candidates that we may file in the future, on clinical hold, requiring us to address any issues raised by the FDA in order to continue the applicable clinical trials.

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 current and potential future 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 data to support clinical development;
- delays in reaching a consensus with regulatory agencies on preclinical and clinical study design;
- inability to obtain appropriate or sufficient test agents or preclinical animal models in connection with the indication the product candidate is meant to address;
- recruiting and retaining the appropriate scientific and technical personnel; and
- the product candidate may not be applicable to the indication we intend to address.

Moreover, even if we obtain positive results from preclinical studies or clinical trials, including interim data from a clinical trial, we may not achieve the same success in ongoing or future trials.

Clinical trials are expensive, difficult to design and implement, 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 clinical trials, including interim data from a clinical trial, of our product candidates may not be predictive of the results of ongoing or future 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 our product candidates for particular indications, including LB-001 for MMA or any other potential indication.

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.

A clinical trial may be suspended or terminated by us, either independently or based on a recommendation by the DSMB, for such trial, by institutional review boards, or 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) findings from inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities; (3) safety issues or adverse side effects; (4) failure to demonstrate a benefit from using a drug; (5) changes in governmental regulations; (6) administrative actions or lack of adequate funding to continue the clinical trial; or (7) imposition of a clinical hold by the FDA or other regulatory authorities. 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."

In January 2020, we announced the submission of an IND for our lead product candidate, LB-001, to support the initiation of a Phase 1/2 clinical trial in pediatric patients with MMA, which the FDA placed on clinical hold in February 2020 pending the resolution of certain clinical and nonclinical questions. FDA lifted this hold in August 2020, and on June 2, 2021, we announced we had dosed the first patient in our Phase 1/2 SUNRISE clinical trial for LB-001 in pediatric patients with MMA. If the FDA places our SUNRISE trial on clinical hold for any reason, including the occurrence of a safety event such as the SAE that occurred in October 2021 described further above and for which we will continue to provide updates to the FDA in accordance with our regulatory obligations, or any similar or other SAEs that may occur in the future, we would be delayed in continuing this study until we address issues raised by the FDA and may not be successful in addressing such issues in order to continue this study. In addition, safety issues that may arise in the SUNRISE trial could adversely affect our ability to develop other product candidates using our proprietary technology.

LB-001 will require extensive clinical testing before we are prepared to submit a Biologic License Application, or BLA, for regulatory approval in the United States or submit a comparable application for regulatory approval in other jurisdictions. 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 or a comparable application for regulatory approval may be approved in other jurisdictions. 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.

Clinical trials are time-consuming and subject to potential delays.

To date, we have not completed any clinical trials for any of our product candidates, including LB-001. On June 2, 2021, we announced that we dosed the first patient in our Phase 1/2 SUNRISE clinical trial. 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. For example, our IND for LB-001 was placed on clinical hold by the FDA in February 2020 in order to evaluate certain clinical and preclinical aspects of the submissions. The clinical hold for LB-001 was lifted in August 2020, and on June 2, 2021, we announced that we dosed the first patient in our Phase 1/2 SUNRISE clinical trial in MMA. However, there can be no assurances that FDA will not place this IND or any clinical trials under this IND, or any IND or clinical trials thereunder relating to other product candidates that we may file in the future on clinical hold, thereby delaying our clinical trials. Clinical trials can be delayed or terminated for a variety of reasons, including delays or failures related to:

- the FDA or other regulatory authorities disagreeing as to the design or implementation of our clinical trials, including alignment with regulatory authorities on registrational endpoints for indications where there are not generally accepted registrational endpoints;
- determining achievable endpoints in connection with our clinical trials that are accepted by the FDA or other regulatory authorities, including with respect to the number of patients and the length of time;
- 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 IRB approval at each site;
- regulators, IRBs, DSMBs, safety committees, or ethics committees, may require that we suspend or terminate our clinical trials for various reasons, including noncompliance with regulatory requirements, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using our product candidate, or a finding that the participants are being exposed to unacceptable health risk;
- recruiting suitable patients to participate in a trial, particularly in light of the COVID-19 pandemic;
- availability of competing therapies and clinical studies, and clinicians' and patients' perceptions as to the potential advantages of the product or product candidate being studied in relation to available therapies or other product candidates in development;
- 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, which could be especially challenging in light of the COVID-19 pandemic;
- 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 SAEs associated with any product candidates we may develop, including those that are viewed to outweigh their potential benefits;
- occurrence of SAEs 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;
- 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 and/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 any sponsor 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 or receive regulatory approval 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 of our 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.

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, particularly in light of the COVID-19 pandemic, 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 clinical 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.

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. Our ability to enroll patients may be significantly delayed by the evolving COVID-19 pandemic, and we do not know the extent and scope of such delays at this point. The enrollment of patients depends on many factors, including:

- the patient inclusion and exclusion criteria defined in the protocol including exclusion criteria;
- 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 and any barriers on patients' ability to travel (such as travel restrictions patients may encounter due to the COVID-19 pandemic);
- the design of the trial;
- availability and efficacy of approved medications for the disease under investigation;
- 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 potentially competitive product candidates being studied in clinical trials and 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;
- the risk that the patients we screen may not be eligible to advance to the clinical trial and that the size of the qualifying patient population;
- perceived risks and benefits of the product candidate under investigation or the method by which such product candidate will be administered to patients, in particular from patients, their caregivers and physicians;
- perceived risks and benefits of genome editing as a therapeutic approach;
- perceived risks and benefits of the product candidate based on any interim data or updates that we may announce;
- 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 and managing relationships with CROs and other vendors, as well as clinical trial sites and investigators;
- 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.

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 gene delivery technologies may negatively impact regulatory approval of, or demand for, our potential products.

We are developing therapies using genome editing and gene therapy technology. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of genome editing and gene therapy for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene editing and gene therapy are unsafe, unethical or immoral, and, consequently, our products may not gain the acceptance of the public or the medical community. Public perception of the cost, durability and potential harm from over-expression of genome editing and gene therapy may also affect public acceptance of our product candidates. 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. The Alliance for Regenerative Medicine in Washington has issued principles setting forth a bioethical framework for the use of gene editing in therapeutic applications that states that the use of gene editing technologies in research that involved altering human embryos or human germline cells is currently not appropriate. Similarly, the National Institute of Health, or 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.

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.

Serious adverse events in our clinical trials, such as the SAE in our Phase 1/2 SUNRISE clinical trial described above, or other clinical trials involving gene delivery technology, particularly AAV genetic medicines, such as candidates based on the same capsid serotypes as our product candidates, or occurring during use of our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. For example, certain gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death, some of which took years to present. SAEs, such as these, whether in our clinical trials or other clinical trials involving gene therapy or genome editing products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity could result in increased regulation and regulatory scrutiny, unfavorable public perception, potential regulatory delays in the testing or approval 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. Other than LB-001, 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 generate sufficient preclinical data to support the initiation of clinical trials;
- 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;
- negative results in the clinical trials for any of our product candidates could negatively impact other product candidates based on the same or similar technologies;
- potential product candidates may not be effective in or applicable to 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 commercially viable;
- we may experience difficulties reaching a consensus with regulatory agencies on preclinical study design;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and/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.

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 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. We or our contract manufacturers must supply all necessary documentation in support of a BLA, or an application for regulatory approval in another jurisdiction, on a timely basis and must adhere to the FDA's, or the applicable regulatory authority's, GLP, and cGMP regulations enforced by the FDA through its facilities inspection program. None of our contract manufacturers has produced a commercially-approved gene editing product, and some of our contract manufacturers have not produced a commercially-approved gene therapy 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, or approval by regulatory authorities in other jurisdictions, 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 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 or regulatory authorities in other jurisdictions 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. In the United States, an alternative manufacturer would need to be qualified through a BLA supplement which could result in further delay, and there may be similar requirements in other jurisdictions. 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.

Although we announced on June 2, 2021 that we dosed the first patient in our first clinical trial, our Phase 1/2 SUNRISE clinical trial for LB-001 in pediatric patients with MMA, 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, some of which took years to present. There can be no assurance that our genome editing technologies will not cause undesirable side effects. In October 2021, a patient in our Phase 1/2 SUNRISE clinical trial experienced an SAE, as described above.

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 regulatory 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 other regulatory authorities, or the IRBs at the institutions in which our studies are conducted, could suspend or terminate our clinical trials or the FDA or other 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 may 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 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.

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, the EMA and other regulatory 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, the EMA and other regulatory 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, the EU or any other jurisdiction until we receive regulatory approval from the applicable regulatory authority. It is possible that the FDA, the EMA or other regulatory authorities may refuse to accept for substantive review any applications for marketing approval 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, the EMA or other regulatory authorities, that such product candidates are safe and effective for their intended uses. The FDA, the EMA and other regulatory authorities 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, the EMA and other regulatory authorities. The FDA, the EMA or other regulatory authorities 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 studies required by regulatory authorities, approval of any marketing application that we submit may be delayed by several years, or may require us to expend significantly more resources than we have available.

Of the large number of potential products in development, only a small percentage successfully completes the FDA, the EMA or regulatory approval processes in other jurisdictions 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, 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 LB-001. 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 authorities, 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, the EMA and regulatory authorities in other jurisdictions.

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 jurisdictions until it receives the requisite approval from such jurisdictions.

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. For example, our IND for LB-001 was placed on clinical hold in February 2020 by the FDA in order to evaluate certain clinical and preclinical aspects of the submissions. FDA lifted this hold in August 2020, and we announced on June 2, 2021 that we dosed the first patient in our Phase 1/2 SUNRISE clinical trial for LB-001 in pediatric patients with MMA. In addition, in October 2021, a patient in our Phase 1/2 SUNRISE clinical trial experienced a serious adverse event, as described further above.

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 jurisdictions 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 and manufacturing 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 are developing 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, EMA 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 distribute a lot until the agency authorizes its release. Slight unplanned 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.

Changes in our manufacturing processes may cause delays in our clinical development and commercialization plans.

The manufacturing processes for our product candidates are complex. As we develop a commercial-scale manufacturing process, we are exploring improvements to the manufacturing process for our product candidates on a continual basis. Changes to the manufacturing process may occur in connection with efforts to optimize our process in preparation for the potential advancement of our clinical trial or commercialization of our product candidates. Changes to this process could induce a change in the purity or potency of a product candidate. In some circumstances, changes in the manufacturing process may require us to perform additional

comparability studies or to collect additional data from patients prior to undertaking additional clinical studies, or to perform a bridging study. The FDA could also require us to file a new IND with respect to such changes in our manufacturing process. These requirements may lead to delays in our clinical development and commercialization plans.

Our employees and independent contractors, including investigators, CROs, CMOs, 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 investigators, CROs, contract manufacturing organizations, or CMOs, 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) GXP; (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.

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

Our computer systems, as well as those of our CROs, CMOs, 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 and those of our third-party contractors, 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 contract with third parties, including those with manufacturing facilities, to supply some of our discovery and preclinical research. We currently also rely on third-party manufacturers and other vendors for the manufacture and testing of our materials for clinical trials. We expect to continue to do so 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 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 such any third-party manufacturer:

- experiencing staffing difficulties;
- undergoing changes in priorities or becoming financially distressed;
- forming relationships with other entities, some of which may be our competitors;
- experiencing unanticipated events that cause negative consequences in such third party's ability to fulfill its obligations to us, including due to the COVID-19 pandemic or natural disasters requiring delay or cessation of operations;
- potential unauthorized disclosure or misappropriation of our intellectual property by CMOs, which may allow our potential competitors to access and exploit our proprietary technology and reduce our trade secret protection;
- having limited capacity for manufacturing slots;
- 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, compliance with specifications, maintaining proper chain of custody, safety, and pharmacovigilance and related reporting.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements in or 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 drug substance or drug product. 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 currently rely, and expect to continue to rely, on CROs and other vendors, as well as clinical trial sites and investigators 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 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.

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, or the EEA, 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, we may be required to repeat clinical trials, which would delay the regulatory approval process. We also are 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 on our business, financial condition, results of operations and prospects.

We have and may in the future 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. For example, in January 2020, we announced a research collaboration with Takeda, in April 2021, we entered into an Exclusive Research Collaboration. License and Option Agreement with CANbridge, and in April 2021, we also entered into a research collaboration with Daiichi. Future collaborators could include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. Under any such arrangements with 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.

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 preclinical studies or clinical trials, provide insufficient funding for a nonclinical program or clinical trial, stop a clinical trial, nonclinical study or abandon a product candidate, repeat or conduct new preclinical studies or clinical trials, or require a new formulation of a product candidate for testing;
- a collaboration partner may seek to renegotiate or terminate its relationship with us due to unsatisfactory clinical or nonclinical 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;
- collaborations involving a co-development relationship increases the risk to successful completion of a development program because it requires coordination between multiple sponsors on a number of topics, including regulatory and development strategy and safety reporting;
- 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.
- in addition, 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.

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 collaborators or strategic partners, such as CANbridge, Daiichi, Takeda, Stanford, NIH, CMRI, the University of Texas or Oregon Health & Science University, or OHSU, 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 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 or sAAVy technologies. Additionally, because our current or future collaborators or 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 or sAAVy technologies. 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, the University of Texas and NIH, 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 and the University of Texas to license our core technology, and we are party to a license agreement with the NIH for development and commercialization rights to the transgene for LB-001. We are also party to an agreement with CMRI for development and commercialization rights to the first capsid produced from the sAAVy platform, sL65. We may enter into additional agreements 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, the University of Texas agreement, the NIH agreement and the CMRI 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, the University of Texas, the NIH, CMRI 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.

Our business has been adversely affected by the ongoing coronavirus pandemic and we expect it to continue to have a negative impact on our business.

In March 2020, the World Health Organization characterized the COVID-19 outbreak a “pandemic.” The pandemic and government measures taken in response have since had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. Although the FDA has approved certain therapies and vaccines for emergency use and distribution, the logistics of implementing a national vaccine program, the willingness of individuals to get vaccinated and the overall efficacy of the vaccines once widely administered, especially as new strains of COVID-19 have been discovered, and the level of resistance these new strains have to the existing vaccines remains unknown.

The Company is following federal, state and local requirements and guidelines with respect to COVID-19, and has allowed its employees to return to working on-premises in accordance with those requirements and guidelines.

Our research, development and manufacturing activities are dependent on our ability to continue our work on premises at our laboratory. We also rely on third parties, such as CROs and CMOs, located in areas that are affected by the COVID-19 pandemic for certain research, development and manufacturing activities. Many of these third parties have also limited their staff from working on premises as part of their response to the COVID-19 pandemic. The COVID-19 pandemic may have a significant negative effect on our business and future results due to a variety of factors, including the health of our employees, our ability to maintain operations, the ability of our third party vendors, suppliers and collaborators to continue operations, any further government and/or public actions taken in response to the pandemic and ultimately the length of the pandemic. The Company is aware that certain of its third-party vendors are being affected by import/export and other restrictions due to COVID-19, which are currently having an impact on certain of the Company’s research, development and manufacturing activities. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company’s business, results of operations and financial position will depend on future developments that are uncertain and cannot be accurately predicted.

Infections and deaths related to COVID-19 have significantly disrupted the United States’ healthcare and healthcare regulatory systems. Such disruptions have diverted healthcare resources away from regular activities at a number of institutions where clinical trials are normally conducted. Depending on the duration and severity of the pandemic, our efforts to engage with potential trial sites in start-up and other activities for our planned clinical trials or other studies may be adversely affected. In addition, other known and

unknown factors caused by COVID-19 could materially delay other aspects of our clinical trials, including our ability to recruit and retain patients and principal investigators and site staff.

The pandemic could also potentially affect the business of the FDA, the EMA or other governmental authorities, which could result in delays in meetings, reviews, inspections and approvals relating to LB-001. Any elongation or de-prioritization of our clinical trials or delay in regulatory review resulting from such disruptions could materially affect the development and study of our product candidates. Further, any decision by the FDA, EMA or other governmental authorities to delay meeting with us or scheduling inspections in light of COVID-19 could have a material adverse effect on our clinical trials, which could increase our operating expenses and have a material adverse effect on our financial results, including the timing and amount of future regulatory milestones we could receive from our partners.

On March 10, 2020 the FDA announced its intention to postpone most inspections of foreign manufacturing facilities and products while local, national and international conditions warrant in response to the COVID-19 pandemic. On March 18, 2020, the FDA announced its intention to temporarily postpone routine surveillance inspections of domestic manufacturing facilities and provided guidance regarding the conduct of clinical trials, which the FDA continues to update. Since then, the FDA has continuously monitored the pandemic and adjusted its inspection policies in line with safety of its inspectors, travel restrictions, and public health needs. For example, as of July 2020, the FDA utilized a rating system to assist in determining when and where it is safest to conduct mission critical domestic and foreign inspections to ensure compliance of manufacturing facilities with FDA quality standards. In May 2021, the FDA issued a roadmap as to how it intended to restore normal inspection operations and reduce the backlog of inspections that had built up due to delays caused by the pandemic. While this roadmap is subject to pandemic developments, the FDA hoped to restore standard operations by September 2021. Depending on the course of the pandemic in various geographic areas, the FDA may not be able to maintain its planned restoration of standard operations and delays or setbacks are possible in the future. Additionally, regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic and may experience delays in their regulatory activities.

Our activities will continue to require a significant expenditure of capital resources. While the full extent of the economic impact brought by and the duration of the COVID-19 pandemic may be difficult to assess or predict, it has resulted in significant disruptions of global financial markets, reducing our ability to access capital. An extended period of disruption in the economy and capital markets could significantly affect our ability to raise additional capital on a timely basis, which would significantly disrupt our programs and also require us to reevaluate our corporate strategy.

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.

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 that protect our business, in whole or in part, or that 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.

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 biosimilar versions of any approved products by submitting abbreviated BLAs 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 to license, develop or commercialize current or future product candidates. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge priority of invention or other features of patentability. Such challenges 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' interests 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 GeneRide platform technology and our sAAVy platform technology.

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 and the NIH, 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 our license agreements, and we expect in our future agreements, we have the right under specified conditions to bring, or permit our licensor(s) 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.

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 for current or future product candidates 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.

Many pharmaceutical companies, biotechnology companies, and academic institutions are competing with us in the fields of genome editing and gene therapy 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' interests 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 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 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 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.

Patent terms 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 Action 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. If we are not able to obtain and enforce market exclusivity for our technologies or product candidates, development and commercialization of our product candidates may be adversely affected.

In our industry, the majority of an innovative product's commercial value is usually realized during the period in which it has market exclusivity. Market exclusivity is comprised of both patent and other intellectual property protection, as well as regulatory exclusivity.

In the United States and some other countries, when market exclusivity expires and generic/biosimilar versions of a product are approved and marketed, there usually are very substantial and rapid declines in the product's sales. Accordingly, our success depends in part on our ability to obtain and maintain patents and other forms of intellectual property rights, including trademarks, trade secrets and in-licenses of intellectual property rights of others, for our product candidates and platform technologies, methods used to manufacture our product candidates, methods of patient stratification and methods for treating patients using our product candidates, as well as our ability to preserve our trade secrets, to prevent third parties from infringing upon our proprietary rights and to operate without infringing upon the proprietary rights of others. Certain research and development activities involved in pharmaceutical and biologics development are exempt from patent infringement in the United States and other jurisdictions, for example, in the U.S. by the provisions of 35 U.S.C. § 271(e)(1), or the Safe Harbor. However, in the United States and certain other jurisdictions, the Safe Harbor exemption is more limited after the sponsor obtains approval for a BLA. Therefore, the risk that a third party might allege patent infringement may increase as our products approach commercialization. We may not be able to apply for patents or obtain patent protection on certain aspects of our product candidates or our platform in a timely fashion or at all. Our existing issued and granted patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technology. There is no guarantee that any of our pending patent applications will result in issued or granted patents, that any of our issued or granted patents will not later be found to be invalid or unenforceable, or that any issued or granted patents will include claims that are sufficiently broad to cover our product candidates, our platform technologies, or any methods relating to them, or to provide meaningful protection from our competitors.

Legal issues related to the patentability of biopharmaceuticals, and methods of their manufacture and use, are complex and uncertain in some countries. In some countries, applicants are not able to protect methods of treating human beings or medical treatment processes. Intellectual property protection varies throughout the world and is subject to change over time. Certain jurisdictions have enacted various rules and laws precluding issuance of patents encompassing any methods a doctor may practice on a human being or any other animal to treat a disease or condition. Thus, in some countries and jurisdictions, it may not be possible to patent some of our product candidates at all. In some countries and jurisdictions, only composition claims may be obtained, and only when those compositions are or contain compounds that are new and/or novel. Also, patents issued with composition claims (i.e., covering product candidates) cannot always be enforced to protect methods of using those compositions to treat or diagnose diseases or medical conditions. In such countries or jurisdictions, enforcement of patents to protect our product candidates, or their uses, may be difficult or impossible. Lack of patent protection in such cases may have a materially adverse effect on our business and financial condition.

Additionally, we expect that some of the product candidates we develop will be regulated as biologics in the United States and therefore they may be subject to competition from biosimilars approved through an abbreviated regulatory pathway. The Affordable Care Act includes a subtitle called the BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-approved 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 approved by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years after the date on which the reference product was first approved.

During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a 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 the other company's product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law. Since the passage of the BPCIA, many states have passed laws or amendments to laws, including laws governing pharmacy practices, which are state-regulated, to regulate the use of biosimilars.

We believe that any of our product candidates approved as a biological product under a BLA should qualify for a 12-year period of regulatory exclusivity under the BPCIA. However, there is a risk 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.

Furthermore, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates, their manufacture or their use might expire before or shortly after those candidates receive regulatory approval and are commercialized. As a result, our owned and licensed patent portfolio 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 where these are available upon regulatory approval in those countries where we are prosecuting patents. This includes in the United States under the Biologics Price Competition and Innovation Act of 2009, or the BPCIA, which permits a patent term extension of up to five years beyond the expiration of the patent. However, the applicable authorities, including the FDA 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 this occurs, our competitors may 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 possible.

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.

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.

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, as well as any future patent reform, and implementation thereof 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. 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 and sAAVy platforms, 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.

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 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 and gene therapy 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 fields of genome editing and 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.

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.

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 genome editing and 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 genome editing and 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;
- 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 government budgets 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. Within the United States, for example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or the Affordable Care Act, significantly revised coverage and payment for health care services. The Affordable Care Act expanded healthcare coverage through Medicaid expansion and the implementation of the individual health insurance mandate and included a number of provisions relevant to coverage and payment of prescription drugs, such as subjecting biologic products to potential competition by lower-cost biosimilars; increasing minimum rebates under the Medicaid Drug Rebate Program and extending the Medicaid Drug Rebate program to prescription drugs covered by Medicaid managed care organizations; requiring manufacturers to provide discounts on Medicare Part D brand name prescription drugs sold to Medicare beneficiaries in the Medicare Part D coverage gap (i.e., the so-called “donut hole”); and subjecting manufacturers to new annual fees for certain branded prescription drugs.

We face uncertainties because there have been, and may be additional, federal legislative and administrative efforts to repeal, substantially modify or invalidate some or all of the provisions of the Affordable Care Act. For example, tax reform legislation was enacted at the end of 2017 that eliminated the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019. As another example, legislation enacted in 2018 increased the discount on Medicare Part D drugs purchased by Medicare beneficiaries in the coverage gap in 2019. The Affordable Care Act has also been subject to judicial challenge. For example, in June 2021, the Supreme Court rejected a challenge to the constitutionality of the Affordable Care Act on the grounds that the states and individuals that brought the challenge did not have standing.

Beyond the Affordable Care Act, there have been ongoing reform efforts that affect pricing or payment for drug products or the healthcare industry more generally. For example, President Biden continues to push for reforms that would address the high cost of drugs. In response to an Executive Order from President Biden, the U.S. Secretary of Health and Human Services recently issued a comprehensive plan for addressing high drug prices that describes a number of legislative approaches and identifies administrative tools to address the high cost of drugs, and Democrats recently included drug pricing reform provisions reflecting elements of the plan in a broader spending package in late 2021, such as capping Medicare Part D patients out-of-pocket costs; establishing penalties for drug prices that increase faster than inflation in Medicare; and authorizing the federal government to negotiate prices on certain, select high cost drugs under Medicare Parts B and D. Additional healthcare reform efforts have sought to address certain issues related to the COVID-19 pandemic, including an expansion of telehealth coverage under Medicare and accelerated or advanced Medicare payments to healthcare providers. Some of these changes have been and may continue to be subject to legal challenge. For example, courts temporarily enjoined a new “most favored nation” payment model for select drugs covered under Medicare Part B that was to take effect on January 1, 2021 and would limit payment based on international drug price and the Centers for Medicare & Medicaid Services subsequently indicated that the rule would not be implemented without further rulemaking. Adoption of new healthcare reform legislation at the federal or state level could affect demand for, or pricing of, our products or product candidates if approved for

sale. We cannot predict, however, the ultimate content, timing or effect of any healthcare reform legislation or action, or its impact on us, and healthcare reform could increase compliance costs and may adversely affect our future business and financial results..

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.

In addition, other legislative changes have been adopted that could have an adverse effect upon, and could prevent, our products' or product candidates' commercial success. More broadly, the Budget Control Act of 2011, as amended, or the Budget Control Act, includes provisions intended to reduce the federal deficit, including reductions in Medicare payments to providers through 2030 (except May 1 to December 31, 2021). The Congressional Budget Office has indicated that the American Rescue Plan Act of 2021 will likely trigger a statutory provision that requires that automatic payment cuts be put into place in 2022 if a statutory action creates a net increase in the deficit and require reductions in Medicare spending. Any significant spending reductions affecting Medicare, Medicaid or other publicly funded or subsidized health programs, or any significant taxes or fees imposed as part of any broader deficit reduction effort or legislative replacement to the Budget Control Act, or otherwise, could have an adverse impact on our anticipated product revenues.

While we have received orphan drug designation for LB-001 for the treatment of MMA, we may not be able to obtain orphan drug marketing exclusivity for LB-001 or enjoy the benefits associated with orphan drug designation.

In July 2019, the FDA granted rare pediatric disease designation for LB-001 for the treatment of MMA, and in April 2019, the FDA granted orphan drug designation for LB-001 for the treatment of MMA. In November 2020, the FDA granted fast track designation for LB-001 for the treatment of MMA, and in June 2021, the European Commission granted orphan drug designation to LB-001 for the treatment of MMA.

Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the United States, orphan drug designation may entitle a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers.

In the European Union, orphan drug designation is intended to promote the development of drugs that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in the European Union and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). In addition, designation is granted for drugs intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers.

Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve a product candidate for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan

designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

There is no guarantee that we will be able to enjoy the benefits associated with orphan drug designation. Further, we may seek orphan drug designation for our other product candidates in the future, however, there is no guarantee that we will obtain any such designation or be able to enjoy the benefits thereof.

Our current and future business operations will be subject to applicable healthcare regulatory laws, which could expose us to penalties and other sanctions.

We are or may become subject to healthcare regulation and enforcement by various government authorities. 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. In the United States, biopharmaceutical companies and their products are subject to extensive regulation at the federal and state level, including laws intended to prevent fraud and abuse in the healthcare industry. These laws, some of which will apply only if and when we have an approved product, include:

- the federal anti-kickback statute makes it illegal for any person or entity to knowingly and willfully, directly or indirectly, solicit, receive, offer, or pay any remuneration that is in exchange for or to induce the referral of business, including the purchase, order, lease of any good, facility, item or service for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. The term "remuneration" has been broadly interpreted to include anything of value;

- federal false claims and false statement laws, including the federal civil False Claims Act, prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs, that are false or fraudulent;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended, which prohibits executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters and which also imposes certain requirements related to the privacy, security and transmission of individually identifiable health information on certain types of entities, which include many healthcare providers and health plans with which we interact;

- the Federal Food, Drug, and Cosmetic Act, which among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products prior to approval or for unapproved indications and regulates the distribution of samples;

- federal laws, including the Medicaid Drug Rebate Program, that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under government healthcare programs; federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and

- the so-called "federal sunshine" law, which requires pharmaceutical and medical device companies to monitor and report certain financial interactions with physicians and teaching hospitals (and additional categories of healthcare practitioners beginning with reports submitted in 2022) to the federal government for re-disclosure to the public.

Also, many state have similar laws and regulations, such as anti-kickback and false claims laws that may be broader in scope and may apply to claims reimbursed by private payors as well as government programs regardless of reimbursement. Additionally, we may be subject to state laws that require pharmaceutical companies to comply with the federal government's and/or pharmaceutical industry's voluntary compliance guidelines, impose specific restrictions on interactions between pharmaceutical companies and healthcare providers or require pharmaceutical companies to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. Other state laws may require pharmaceutical companies to file reports relating to pricing and marketing information and state and local laws that require the registration of pharmaceutical sales representatives. Finally, there are state laws governing the privacy and security of health information, many of which differ from each other in significant ways and often are not preempted by HIPAA. Many of these laws and regulations also contain ambiguous requirements or require administrative guidance for implementation.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Given the breadth of the laws and regulations, limited guidance for certain laws and regulations and evolving government interpretations of the laws and regulations, governmental authorities may possibly conclude that our business practices may not comply with such laws. 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. 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.

There are also similar healthcare laws and regulations in the European Union and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers. 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 publicly 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.

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 CMOs and other third party vendors 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.

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.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies and contractual obligations could adversely affect our business.

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

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. For example, the HIPAA and its implementing regulations establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity

and availability of electronic protected health information. While we have determined that we are neither a "covered entity" nor a "business associate" directly subject to HIPAA, many of the U.S. health care providers, including U.S. clinical trial sites, with which we interact are subject to HIPAA, and we have assumed contractual obligations related to protecting the privacy of personal information. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. If we are unable to properly protect the privacy and security of protected health information, we could be found to have breached our contracts and we could face civil and criminal penalties. In addition, our operations have been affected by the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020. The CCPA gives California consumers (defined to include all California residents) certain rights, including the right to ask covered companies to disclose the types of personal information collected, the categories of sources from which such information was collected, the business purpose for collecting or selling the consumer's personal information, the categories of third parties with whom a covered company shares personal information, and specific pieces of information collected by a covered company. The CCPA imposes several obligations on covered companies to provide notice to California consumers regarding their data processing activities. The CCPA also gives California consumers the right to ask covered companies to delete a consumer's personal information and it places limitations on a covered company's ability to sell personal information, including providing consumers a right to opt out of sales of their personal information. On November 3, 2020, California voters passed a ballot initiative approving the California Privacy Rights Act, or CPRA, which will significantly expand the CCPA to incorporate additional provisions, including a requirement that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA will also expand personal information rights of California residents, including creating a right to opt out of sharing of personal information with third parties for advertising, expanding the lookback period for the right to know about personal information held by businesses, and expanding the right to erasure for information held by third parties. Most CPRA provisions will take effect on January 1, 2023, though the obligations will apply to any personal information collected after January 1, 2022. Other states are also enacting comprehensive privacy legislation, including Virginia and Colorado, both of which passed expansive privacy laws in 2021 that take effect in 2023.

In addition, we may be subject to privacy and security laws in the various jurisdictions outside of the United States in which we operate, obtain or store personally identifiable information. The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. For example, the processing of personal data in the EEA is subject to the General Data Protection Regulation, or the GDPR, which took effect in May 2018. The GDPR increases obligations with respect to clinical trials conducted in the EEA, such as in relation to the provision of fair processing notices, responding to data subjects who exercise their rights and reporting certain data breaches to regulators and affected individuals. The GDPR also requires us to enter certain contractual arrangements with third parties that process GDPR-covered personal data on our behalf. The GDPR also increases the scrutiny applied to transfers of personal data from the EEA (including from clinical trial sites in the EEA) to countries that are considered by the European Commission to lack an adequate level of data protection, such as the United States. The July 2020 invalidation by the Court of Justice of the European Union of the EU-U.S. Privacy Shield framework, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the United States, has led to increased scrutiny on data transfers from the EEA to the United States generally and may increase our costs of compliance with data privacy legislation. If our or our partners' or service providers' privacy or data security measures fail to comply with the GDPR requirements, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and/or fines of up to 20 million Euros or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, as well as claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill.

Data privacy remains an evolving landscape at both the domestic and international level, with new regulations coming into effect and continued legal challenges, and our ongoing efforts to comply with evolving laws and regulations may be costly and require ongoing modifications to our policies, procedures and systems. Our efforts to comply may also be unsuccessful. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our practices. Failure to comply with laws regarding data protection would expose us to risk of enforcement actions taken by data protection authorities in the EU and elsewhere and carries with it the potential for significant penalties if we are found to be non-compliant as well as private lawsuits by affected data subjects. Similarly, failure to comply with federal and state laws in the United States regarding privacy and security of personal information could expose us to penalties under such laws and private lawsuits brought by data subjects. Any such failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages by data subjects, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to retain 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 Frederic Chereau, our President and Chief Executive Officer, as well as the other members of our management, scientific, clinical, technical 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 subjects us to a number of risks, including the risks of impeding the achievement of our research, development and commercialization objectives, failure to coordinate responsibilities and tasks, the impact on corporate culture, and the retention of historical knowledge. Further, our Board of Directors and management succession planning efforts may not be effective, which could adversely impact our business.

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, as well as 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 may encounter difficulties in managing our growth, which could disrupt our operations.

We have experienced significant growth recently. Our management has diverted, and may need to divert, a disproportionate amount of its attention away from our day-to-day activities to devote time to managing our growth. To manage our growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such growth, we may not be able to effectively manage the expansion of our operations or train the 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. Managing our 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 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.

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 using CRISPR/Cas9, TALENs, meganucleases, Mega-TALs and ZFNs, including bluebird bio, Caribou Biosciences, CRISPR Therapeutics, Editas Medicine, Intellia Therapeutics, Sangamo Therapeutics, Precision BioSciences and Graphite Bio. We may also compete with companies developing gene therapy products, including Homology Medicines, Astellas Pharma, Pfizer, bluebird bio, uniQure and Generation Bio. There are also companies pursuing base editing technologies, including Beam 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 an AAV gene therapy, and HemoShear Therapeutics using a small molecule. If any of our competitors obtains regulatory approval for a treatment for MMA, it could negatively affect our ability to successfully commercialize LB-001, if approved.

There are several companies developing competing products using AAV gene therapies that target CN, the indication for which we conducted development activities for LB-301. These organizations include Genethon, Selecta Biosciences and the Institute for Life Changing Medicines. If any of our competitors obtain regulatory approval for a treatment for CN, it could negatively affect our ability to successfully commercialize LB-301, 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.

The successful commercialization of our product candidates will depend in part on the extent to which third-party payors, including government healthcare programs as well as private health insurance, establish favorable coverage and reimbursement for our product candidates, if approved.

The availability and adequacy of coverage and reimbursement by third-party payors, including governmental healthcare programs such as Medicare and Medicaid, as well as private health insurance, will likely be essential for most patients to be able to afford our product candidates, assuming regulatory approval. Our initial target patient populations are relatively small and costs associated with the development and commercialization of our genomic medicine product candidates are substantial. We therefore expect the cost of a single administration of our products to be significant if and when approved. Our ability to achieve acceptable levels of coverage and reimbursement for such products and services required to administer such products by third-party payors will likely have a significant impact 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.

There is significant uncertainty related to third party payor 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. Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could 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. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. We therefore cannot predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates. Even if we obtain coverage and adequate reimbursement for our product candidates with respect to a particular third party payor, there is no certainty that coverage will be maintained or reimbursement rates will not change.

Within the United States, no uniform policy for coverage and reimbursement for products exists among third-party payors. 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. Third-party payors increasingly are limiting coverage and utilization of pharmaceutical products and challenging prices charged for pharmaceutical products and services. Assuming we obtain coverage for a product by a third-party payor, the third-party payor may implement utilization management controls, such as requiring pre-approval before our product will be covered for a particular patient, which may limit access to our product. In addition, the reimbursement rates may not be adequate or may require co-payments that patients find unacceptably high. Net prices for our products may be reduced by mandatory discounts or rebates that we are required to provide to certain government healthcare programs or private payors or by discounts we negotiate with third party payors. In addition, given the novel nature of our product candidates, we may need to develop new reimbursement models in order to realize adequate value. Even if we are able to develop such models, 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 coverage is limited, access to our products is subject to utilization management controls or reimbursement is inadequate, 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.

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.

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 currently 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;
- 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, many of the individual countries in Europe and other countries outside of Europe with which we will need to comply. Many U.S.-based biotechnology companies have found the process of marketing their own products outside the United States 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 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.

Risks Related to Our Common Stock and Indebtedness

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 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;
- results from 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.

The terms of our Loan and Security Agreement place restrictions on our operating and financial flexibility.

In July 2019, we entered into the Loan Agreement, under which term loans in an aggregate principal amount of $20.0 million were made available to us in two tranches, subject to certain terms and conditions. As of September 30, 2021, we had drawn down the $10.0 million first tranche. In the second quarter of 2021, we met the conditions to initiate drawdown of the $10.0 million second tranche but did not exercise our right to do so and the option to draw down the second tranche of the Term Loans expired. See Note 6 to our condensed financial statements found elsewhere in this quarterly report on Form 10-Q. The Loan Agreement contains representations and warranties, affirmative and negative covenants applicable to us and our subsidiaries and events of default, as more fully described in the Loan Agreement. The affirmative covenants include, among others, covenants requiring us and our subsidiaries to maintain our legal existence and material governmental approvals, deliver certain financial reports, maintain insurance coverage and maintain certain cash balances in controlled accounts. The negative covenants include, among others, restrictions on dispositions, changes in business, management, ownership or business locations, mergers or acquisitions, indebtedness, encumbrances, maintenance of collateral accounts, distributions, investments, transactions with affiliates and subordinated debt.

The Loan Agreement also includes events of default, the occurrence and continuation of which provide Oxford Finance LLC, as collateral agent, with the right to exercise remedies against us and the collateral securing the loans under the Loan Agreement, including foreclosure against our properties securing the Loan Agreement, including our cash, potentially requiring us to renegotiate our agreement on terms less favorable to us or to immediately cease operations. These events of default include, among other things, the nonpayment of principal or interest, violations of covenants, material adverse changes, attachment, levy, restraint on business, cross-defaults on material indebtedness, bankruptcy, material judgments, misrepresentations, subordinated debt, governmental approvals, lien priority and delisting.

Further, if we are liquidated, the Lenders’ right to repayment would be senior to the rights of the holders of our common stock to receive any proceeds from the liquidation. The lenders could declare a default upon the occurrence of any event that they interpret as a material adverse change as defined under the Loan Agreement, thereby requiring us to repay the loan immediately or to attempt to reverse the declaration of default through negotiation or litigation. Any declaration by the lenders of an event of default could significantly harm our business and prospects and could cause the price of our common stock to decline.

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, 2021, we believe our executive officers and directors, combined with our stockholders who owned more than 5% of our common stock, together with their respective affiliates, beneficially owned a significant percentage of our outstanding common stock, including shares subject to outstanding options that are exercisable within 60 days after such date. 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.

We are an "emerging growth company" and a "smaller reporting company," and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting 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 December 31, 2023. 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 December 31, 2023, 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 December 31, 2023. 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.

Further, even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company," which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. In addition, if we are a smaller reporting company, we would not be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, or Section 404. These exemptions and reduced disclosures in our SEC filings due to our status as a smaller reporting company may make it harder for investors to analyze our results of operations and financial prospects. Investors may find our common stock less attractive because we may 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 prices may be more volatile.

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;
- 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.

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.

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.

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, File No. 001-38707, 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, File No. 001-38707, filed on October 29, 2018).
EXHIBIT 10.1*	—	Form of Indemnification Agreement applicable to directors and executive officers.
EXHIBIT 10.2*	—	Form of Inducement Award Agreement.
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*	—	Inline XBRL Instance Document. The instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
EXHIBIT 101.SCH*	—	Inline XBRL Taxonomy Extension Schema Document.
EXHIBIT 101.CAL*	—	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
EXHIBIT 101.DEF*	—	Inline XBRL Taxonomy Extension Definition Linkbase Document.
EXHIBIT 101.LAB*	—	Inline XBRL Taxonomy Extension Label Linkbase Document.
EXHIBIT 101.PRE*	—	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
EXHIBIT 104*	—	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith.

** Furnished herewith.

\+ Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

† Indicates a management contract or compensatory plan.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

LogicBio Therapeutics, Inc.

Dated: November 15, 2021

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

Dated: November 15, 2021

By: /s/ Cecilia Jones
Cecilia Jones
Chief Financial Officer

Exhibit 10.1

INDEMNIFICATION AGREEMENT

THIS INDEMNIFICATION AGREEMENT (the "**Agreement**") is made and entered into as of [_____], 2021, between LogicBio Therapeutics, Inc., a Delaware corporation (the "**Company**"), and [_____] ("**Indemnitee**"), and this Agreement shall be deemed effective from and after the date on which the Indemnitee became [a director] [an officer] of the Corporation (the "**Effective Date**").

WITNESSETH THAT:

WHEREAS, highly competent persons have become more reluctant to serve corporations as directors, officers or in other capacities unless they are provided with adequate protection through insurance or adequate indemnification against inordinate risks of claims and actions against them arising out of their service to and activities on behalf of the corporation;

WHEREAS, the Board of Directors of the Company (the "**Board**") has determined that, in order to attract and retain qualified individuals, the Company will attempt to maintain on an ongoing basis, at its sole expense, liability insurance to protect persons serving the Company and its subsidiaries from certain liabilities. Although the furnishing of such insurance has been a customary and widespread practice among United States-based corporations and other business enterprises, the Company believes that, given current market conditions and trends, such insurance may be available to it in the future only at higher premiums and with more exclusions. At the same time, directors, officers and other persons in service to corporations or business enterprises are being increasingly subjected to expensive and time-consuming litigation relating to, among other things, matters that traditionally would have been brought only against the Company or business enterprise itself. The Amended and Restated Bylaws of the Company (as amended and/or restated from time to time, the "**Bylaws**") require indemnification of the directors, officers and any person who at the request of the Company is or was serving as a director, officer, employee, member, trustee or agent of another corporation or of a partnership, joint venture, trust, nonprofit entity or other enterprise (including service with respect to employee benefit plans) against all liability and loss suffered. Indemnitee may also be entitled to indemnification pursuant to the General Corporation Law of the State of Delaware ("**DGCL**"). The Bylaws and the DGCL expressly provide that the indemnification provisions set forth therein are not exclusive, and thereby contemplate that contracts may be entered into between the Company and members of the Board, officers and other persons with respect to indemnification;

WHEREAS, the uncertainties relating to such liability insurance and to indemnification have increased the difficulty of attracting and retaining such persons;

WHEREAS, the Board has determined that the increased difficulty in attracting and retaining such persons is detrimental to the best interests of the Company's stockholders and that the Company should act to assure such persons that there will be increased certainty of such protection in the future;

WHEREAS, it is reasonable, prudent and necessary for the Company contractually to obligate itself to indemnify, and to advance expenses on behalf of, such persons to the fullest extent permitted by applicable law so that they will serve or continue to serve the Company free from undue concern that they will not be so indemnified;

WHEREAS, this Agreement is a supplement to and in furtherance of the Bylaws and any resolutions adopted pursuant thereto, as well as any rights of Indemnitee under any directors' and officers' liability insurance policy, and shall not be deemed a substitute therefor, nor to diminish or abrogate any rights of Indemnitee thereunder;

WHEREAS, Indemnitee does not regard the protection available under the Bylaws and insurance as adequate in the present circumstances, and may not be willing to serve as a director, officer or otherwise without adequate protection, and the Company desires Indemnitee to serve in such capacity. Indemnitee is willing to serve, continue to serve and to take on additional service for or on behalf of the Company on the condition that Indemnitee be so indemnified; and

WHEREAS, Indemnitee may have certain rights to indemnification and/or insurance provided by other entities and/or organizations which Indemnitee and such other entities and/or organizations intend to be secondary to the primary obligation of the Company to indemnify Indemnitee as provided herein, with the Company's acknowledgement and agreement to the foregoing being a material consideration to Indemnitee's willingness to serve on the Board.

NOW, THEREFORE, in consideration of Indemnitee's agreement to serve as [a director][an officer] at the request of the Company from and after the Effective Date, the parties hereto agree as follows:

Indemnity of Indemnitee. 1. The Company hereby agrees to hold harmless and indemnify Indemnitee to the fullest extent permitted by law, as such may be amended from time to time. In furtherance of the foregoing indemnification, and without limiting the generality thereof:

(a) Proceedings Other Than Proceedings by or in the Right of the Company. Indemnitee shall be entitled to the rights of indemnification provided in this Section 1(a) if, by reason of Indemnitee's Corporate Status (as hereinafter defined), Indemnitee is, or is threatened to be made, a party to or participant in any Proceeding (as hereinafter defined) other than a Proceeding by or in the right of the Company. Pursuant to this Section 1(a), Indemnitee shall be indemnified against all Expenses (as hereinafter defined), judgments, penalties, fines and amounts paid in settlement (including all interest, assessments and other charges paid or payable in connection with or in respect of such Expenses, judgments, penalties, fines and amounts paid in settlement) actually and reasonably incurred by Indemnitee or on Indemnitee's behalf, in connection with such Proceeding or any claim, issue or matter therein, if Indemnitee acted in good faith and in a manner Indemnitee reasonably believed to be in or not opposed to the best interests of the Company, and with respect to any criminal Proceeding, had no reasonable cause to believe Indemnitee's conduct was unlawful. The parties hereto intend that this Agreement shall provide to the fullest extent permitted by law for indemnification in excess of that expressly permitted by statute, including, without limitation, any indemnification provided by the Certificate of Incorporation (as hereinafter defined), the Bylaws, vote of the Company's stockholders or Disinterested Directors (as hereinafter defined) or applicable law.

(b) Proceedings by or in the Right of the Company. Indemnitee shall be entitled to the rights of indemnification provided in this Section 1(b) if, by reason of Indemnitee's Corporate Status, Indemnitee is, or is threatened to be made, a party to or participant in any Proceeding brought by or in the right of the Company. Pursuant to this Section 1(b), Indemnitee shall be indemnified to the fullest extent permitted by applicable law against all Expenses actually and reasonably incurred by Indemnitee, or on Indemnitee's behalf, in connection with such Proceeding or any claim, issue or matter therein, if Indemnitee acted in good faith and in a manner Indemnitee reasonably believed to be in or not opposed to the best interests of the Company. No indemnification for such Expenses shall be made under this Section 1(b) in respect of any claim, issue or matter as to which Indemnitee shall have been finally adjudged to be liable to the Company unless and only to the extent that the Court of Chancery of the State of Delaware (the "**Delaware Court**") or any court in which the Proceeding was brought shall determine upon application that, despite the adjudication of liability but in view of all the circumstances of the case, Indemnitee is fairly and reasonably entitled to such indemnification for such Expenses as the Delaware Court or other court shall deem proper.

(c) Indemnification for Expenses of a Party Who is Wholly or Partly Successful. Notwithstanding any other provision of this Agreement, to the fullest extent permitted by applicable law and to the extent that Indemnitee is, by reason of Indemnitee's Corporate Status, a party to and is successful, on the merits or otherwise, in any Proceeding, Indemnitee shall be indemnified to the maximum extent permitted by law, as such may be amended from time to time, against all Expenses actually and reasonably incurred by or on behalf of Indemnitee in connection therewith. If Indemnitee is not wholly successful in such Proceeding but is successful, on the merits or otherwise, as to one or more but less than all claims, issues or matters in such Proceeding, the Company shall indemnify Indemnitee against all Expenses actually and reasonably incurred by or on behalf of Indemnitee in connection with each successfully resolved claim, issue or matter to the fullest extent permitted by law. For purposes of this Section 1(c) and without limitation, the termination of any claim, issue or matter in such a Proceeding by dismissal, with or without prejudice, shall be deemed to be a successful result as to such claim, issue or matter.

Additional Indemnity. 2. In addition to, and without regard to any limitations on, the indemnification provided for in Section 1 of this Agreement, the Company shall and hereby does indemnify and hold harmless Indemnitee to the fullest extent permitted by applicable law against all Expenses, judgments, penalties, fines and amounts paid in settlement (including all interest, assessments and other charges paid or payable in connection with or in respect of such Expenses, judgments, penalties, fines and amounts paid in settlement) actually and reasonably incurred by Indemnitee or on Indemnitee's behalf if, by reason of Indemnitee's Corporate Status, Indemnitee is, or is threatened to be made, a party to or participant in any Proceeding (including a Proceeding by or in the right of the Company), including, without limitation, all liability arising out of the negligence or active or passive wrongdoing of Indemnitee. The only limitation that shall exist upon the Company's obligations pursuant to this Agreement shall be that the Company shall not be obligated to make any payment to Indemnitee that is finally determined (under the procedures, and subject to the presumptions, set forth in Sections 6 and 7 hereof) to be unlawful.

Contribution. 3.

(a) Whether or not the indemnification provided in Sections 1 and 2 hereof is available, in respect of any threatened, pending or completed action, suit or proceeding in which the Company is jointly liable with Indemnitee (or would be if joined in such action, suit or proceeding), the Company shall pay, in the first instance, the entire amount of any judgment or settlement of such action, suit or proceeding, without requiring Indemnitee to contribute to such payment and the Company hereby waives and relinquishes any right of contribution it may have against Indemnitee. The Company shall not enter into any settlement of any Proceeding in which the Company is jointly liable with Indemnitee (or would be if joined in such Proceeding) unless such settlement provides for a full and final release of all claims asserted against Indemnitee.

(b) Without diminishing or impairing the obligations of the Company set forth in Section 3(a), if, for any reason, Indemnitee shall elect or be required to pay all or any portion of any judgment, penalty, fine or settlement in any threatened, pending or completed action, suit or proceeding in which the Company is jointly liable with Indemnitee (or would be if joined in such action, suit or proceeding), the Company shall contribute to the amount of Expenses, judgments, penalties, fines and amounts paid in settlement actually and reasonably incurred and paid or payable by Indemnitee in proportion to the relative benefits received by the Company and all officers, directors or employees of the Company, other than Indemnitee, who are jointly liable with Indemnitee (or would be if joined in such action, suit or proceeding), on the one hand, and Indemnitee, on the other hand, from the transaction or events from which such action, suit or proceeding arose; provided, however, that the proportion determined on the basis of relative benefit may, to the extent necessary to conform to law, be further adjusted by reference to the relative fault of the Company and all officers, directors or employees of the Company other than Indemnitee who are jointly liable with Indemnitee (or would be if joined in such action, suit or proceeding), on the one hand, and Indemnitee, on the other hand, in connection with the transaction or events that resulted in such Expenses, judgments, penalties, fines or settlement amounts, as well as any other equitable considerations which applicable law may require to be considered. The relative fault of the Company and all officers, directors or employees of the Company, other than Indemnitee, who are jointly liable with Indemnitee (or would be if joined in such action, suit or proceeding), on the one hand, and Indemnitee, on the other hand, shall be determined by reference to, among other things, the degree to which their actions were motivated by intent to gain personal profit or advantage, the degree to which their liability is primary or secondary and the degree to which their conduct is active or passive.

(c) The Company hereby agrees to fully indemnify and hold Indemnitee harmless from any claims for contribution which may be brought by officers, directors or employees of the Company, other than Indemnitee, who may be jointly liable with Indemnitee.

(d) To the fullest extent permissible under applicable law, if the indemnification provided for in this Agreement is unavailable to Indemnitee for any reason whatsoever, the Company, in lieu of indemnifying Indemnitee, shall contribute to the amount incurred by Indemnitee, whether for judgments, fines, penalties, excise taxes, amounts paid or to be paid in settlement and/or for Expenses, in connection with any claim relating to an indemnifiable event under this Agreement, in such proportion as is deemed fair and reasonable in light of all of the circumstances of such Proceeding in order to reflect (i) the relative benefits received by the Company and Indemnitee as a result of the event(s) and/or transaction(s) giving cause to such Proceeding; and/or (ii) the relative fault of the Company (and its directors, officers, employees and agents) and Indemnitee in connection with such event(s) and/or transaction(s).

Indemnification for Expenses of a Witness. 4. Notwithstanding any other provision of this Agreement, to the fullest extent permitted by applicable law and to the extent that Indemnitee is, by reason of Indemnitee's Corporate Status, a witness, or is made (or asked) to respond to discovery requests, in any Proceeding to which Indemnitee is not a party, Indemnitee shall be indemnified against all Expenses actually and reasonably incurred by Indemnitee or on Indemnitee's behalf in connection therewith.

Advancement of Expenses. 5. Notwithstanding any other provision of this Agreement, Indemnitee shall, in all events, control the defense of Indemnitee in any Proceeding (or any part of any Proceeding) by reason of Indemnitee's Corporate Status, and the Company shall advance all Expenses incurred by or on behalf of Indemnitee in connection with any Proceeding (x) not initiated by Indemnitee or (y) initiated by Indemnitee with the prior approval of the Board as provided in Section 6(b), and such advancement will be made within thirty (30) days after the receipt by the Company of a statement or statements from Indemnitee requesting such advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably

evidence the Expenses incurred by Indemnitee and shall include or be preceded or accompanied by a written undertaking by or on behalf of Indemnitee to repay any Expenses advanced if it shall ultimately be determined that Indemnitee is not entitled to be indemnified against such Expenses. Any advances and undertakings to repay pursuant to this Section 5 shall be unsecured and interest free, and shall be made without regard to Indemnitee's ability to repay the Expenses and without regard to Indemnitee's ultimate entitlement to indemnification under the other provisions of this Agreement. Advances shall include any and all reasonable Expenses incurred pursuing an action to enforce this right of advancement, including Expenses incurred preparing and forwarding statements to the Company to support the advances claimed. Indemnitee shall qualify for advances upon the execution and delivery to the Company of this Agreement, which shall constitute an undertaking providing that Indemnitee undertakes to repay the amounts advanced (without interest) to the extent that it is ultimately determined that Indemnitee is not entitled to be indemnified by the Company. No other form of undertaking shall be required other than the execution of this Agreement.

6. Procedures and Presumptions for Determination of Entitlement to Indemnification. It is the intent of this Agreement to secure for Indemnitee rights of indemnity that are as favorable as may be permitted under the DGCL and public policy of the State of Delaware. Accordingly, the parties agree that the following procedures and presumptions shall apply in the event of any question as to whether Indemnitee is entitled to indemnification under this Agreement:

(a) To obtain indemnification under this Agreement, Indemnitee shall submit to the Company a written request, including therein or therewith such documentation and information as is reasonably available to Indemnitee and is reasonably necessary to determine whether and to what extent Indemnitee is entitled to indemnification. The Secretary of the Company shall, promptly upon receipt of such a request for indemnification, advise the Board in writing that Indemnitee has requested indemnification. Notwithstanding the foregoing, any failure of Indemnitee to provide such a request to the Company, or to provide such a request in a timely fashion, shall not relieve the Company of any liability that it may have to Indemnitee unless, and to the extent that, such failure actually and materially prejudices the interests of the Company.

(b) Upon written request by Indemnitee for indemnification pursuant to the first sentence of Section 6(a) hereof, a determination with respect to Indemnitee's entitlement thereto shall be made in the specific case: (i) if a Change in Control shall have occurred, by Independent Counsel (as hereinafter defined) in a written opinion to the Board, a copy of which shall be delivered to Indemnitee, (ii) if a Change in Control shall not have occurred, by one of the following four methods, which shall be at the election of the Board: (1) by a majority vote of the Disinterested Directors (as hereinafter defined), even if less than a quorum, (2) by a committee of Disinterested Directors designated by a majority vote of the Disinterested Directors, even if less than a quorum, (3) if there are no Disinterested Directors or if the Disinterested Directors so direct, by Independent Counsel in a written opinion to the Board, a copy of which shall be delivered to Indemnitee, or (4) if so directed by the Board, by the stockholders of the Company.

(c) If the determination of entitlement to indemnification is to be made by Independent Counsel pursuant to Section 6(b) hereof, the Independent Counsel shall be selected as provided in this Section 6(c). If a Change in Control shall not have occurred, the Independent Counsel shall be selected by the Board, and the Company shall give written notice to Indemnitee advising Indemnitee of the identity of the Independent Counsel so selected. If a Change in Control shall have occurred, the Independent Counsel shall be selected by Indemnitee (unless Indemnitee shall request that such selection be made by the Board, in which event the preceding sentence shall apply), and Indemnitee shall give written notice to the Company advising it of the identity of the Independent Counsel so selected. In either event, Indemnitee or the Company, as the case may be, may, within ten (10) days after such written notice of selection shall have been given, deliver to the Company or to Indemnitee, as the case may be, a written objection to such selection; provided, however, that such objection may be asserted only on the ground that the Independent Counsel so selected does not meet the requirements of "Independent Counsel" as defined in Section 13 of this Agreement, and the objection shall set forth with particularity the factual basis of such assertion. Absent a proper and timely objection, the person so selected shall act as Independent Counsel. If a written objection is made and substantiated, the Independent Counsel selected may not serve as Independent Counsel unless and until such objection is withdrawn or the Delaware Court has determined that such objection is without merit. If, within twenty (20) days after the later of submission by Indemnitee of a written request for indemnification pursuant to Section 6(a) hereof and the final disposition of the Proceeding, no Independent Counsel shall have been selected and not objected to, either the Company or Indemnitee may petition the Delaware Court for resolution of any objection which shall have been made by the Company or Indemnitee to the Company's selection of Independent Counsel and/or

for the appointment as Independent Counsel of a person selected by the court or by such other person as the court shall designate, and the person with respect to whom all objections are so resolved or the person so appointed shall act as Independent Counsel under Section 6(b) hereof. The Company shall pay any and all reasonable fees and expenses of Independent Counsel incurred by such Independent Counsel in connection with acting pursuant to Section 6(b) hereof, and the Company shall pay all reasonable fees and expenses incident to the procedures of this Section 6(c), regardless of the manner in which such Independent Counsel was selected or appointed.

(d) In making a determination with respect to entitlement to indemnification or advance of Expenses hereunder, the person or persons or entity making such determination shall presume that Indemnitee is entitled to indemnification under this Agreement. Anyone seeking to overcome this presumption shall have the burden of proof and the burden of persuasion by clear and convincing evidence. Neither the failure of the Company (including by its directors or Independent Counsel) to have made a determination prior to the commencement of any action pursuant to this Agreement that indemnification is proper in the circumstances because Indemnitee has met the applicable standard of conduct, nor an actual determination by the Company (including by its directors or Independent Counsel) that Indemnitee has not met such applicable standard of conduct, shall be a defense to the action or create a presumption that Indemnitee has not met the applicable standard of conduct.

(e) Indemnitee shall be deemed to have acted in good faith if Indemnitee's action is based on the records or books of account of the Enterprise (as hereinafter defined), including financial statements, or on information supplied to Indemnitee by the officers of the Enterprise in the course of their duties, or on the advice of legal counsel for the Enterprise or on information or records given or reports made to the Enterprise by an independent certified public accountant or by an appraiser or other expert selected with reasonable care by the Enterprise. In addition, the knowledge and/or actions, or failure to act, of any director, officer, agent or employee of the Enterprise shall not be imputed to Indemnitee for purposes of determining the right to indemnification under this Agreement. Whether or not the foregoing provisions of this Section 6(e) are satisfied, it shall in any event be presumed that Indemnitee has at all times acted in good faith and in a manner Indemnitee reasonably believed to be in or not opposed to the best interests of the Company. Anyone seeking to overcome this presumption shall have the burden of proof and the burden of persuasion by clear and convincing evidence.

(f) If the person, persons or entity empowered or selected under Section 6 to determine whether Indemnitee is entitled to indemnification shall not have made a determination within sixty (60) days after receipt by the Company of the request therefor, the requisite determination of entitlement to indemnification shall be deemed to have been made and Indemnitee shall be entitled to such indemnification absent (i) a misstatement by Indemnitee of a material fact, or an omission of a material fact necessary to make Indemnitee's statement not materially misleading, in connection with the request for indemnification, or (ii) a prohibition of such indemnification under applicable law; provided, however, that such 60-day period may be extended for a reasonable time, not to exceed an additional thirty (30) days, if the person, persons or entity making such determination with respect to entitlement to indemnification in good faith requires such additional time to obtain or evaluate documentation and/or information relating thereto; and provided, further, that the foregoing provisions of this Section 6(f) shall not apply if the determination of entitlement to indemnification is to be made by the stockholders pursuant to Section 6(b) of this Agreement and if (A) within fifteen (15) days after receipt by the Company of the request for such determination, the Board or the Disinterested Directors, if appropriate, resolve to submit such determination to the stockholders for their consideration at an annual meeting thereof to be held within seventy-five (75) days after such receipt and such determination is made thereat, or (B) a special meeting of stockholders is called within fifteen (15) days after such receipt for the purpose of making such determination, such meeting is held for such purpose within sixty (60) days after having been so called and such determination is made thereat.

(g) Indemnitee shall cooperate with the person, persons or entity making such determination with respect to Indemnitee's entitlement to indemnification, including providing to such person, persons or entity upon reasonable advance request any documentation or information which is not privileged or otherwise protected from disclosure and which is reasonably available to Indemnitee and reasonably necessary to such determination. Any Independent Counsel, member of the Board or stockholder of the Company shall act reasonably and in good faith in making a determination regarding Indemnitee's entitlement to indemnification under this Agreement. Any costs or expenses (including attorneys' fees and disbursements) incurred by Indemnitee in so cooperating with the person, persons or entity making such determination shall be borne by the Company (irrespective of the determination as to Indemnitee's entitlement to indemnification) and the Company hereby indemnifies and agrees to hold Indemnitee harmless therefrom.

(b) The Company acknowledges that a settlement or other disposition short of final judgment may be successful if it permits a party to avoid expense, delay, distraction, disruption and uncertainty. In the event that any action, suit or proceeding to which Indemnitee is a party is resolved in any manner other than by adverse judgment against Indemnitee (including, without limitation, settlement of such action, suit or proceeding with or without payment of money or other consideration) it shall be presumed that Indemnitee has been successful on the merits or otherwise in such action, suit or proceeding. Anyone seeking to overcome this presumption shall have the burden of proof and the burden of persuasion by clear and convincing evidence.

(c) The termination of any Proceeding or of any claim, issue or matter therein, by judgment, order, settlement or conviction, or upon a plea of nolo contendere or its equivalent, shall not (except as otherwise expressly provided in this Agreement) of itself adversely affect the right of Indemnitee to indemnification or create a presumption that Indemnitee did not act in good faith and in a manner which Indemnitee reasonably believed to be in or not opposed to the best interests of the Company or, with respect to any criminal Proceeding, that Indemnitee had reasonable cause to believe that Indemnitee's conduct was unlawful.

Remedies of Indemnitee.7.

(a) In the event that (i) a determination is made pursuant to Section 6 of this Agreement that Indemnitee is not entitled to indemnification under this Agreement, (ii) advancement of Expenses is not timely made pursuant to Section 5 of this Agreement, (iii) no determination of entitlement to indemnification is made pursuant to Section 6(b) of this Agreement within ninety (90) days after receipt by the Company of the request for indemnification, (iv) payment of indemnification is not made pursuant to this Agreement within ten (10) days after receipt by the Company of a written request therefor or (v) payment of indemnification is not made within ten (10) days after a determination has been made that Indemnitee is entitled to indemnification or such determination is deemed to have been made pursuant to Section 6 of this Agreement, Indemnitee shall be entitled to an adjudication in an appropriate court of the State of Delaware, or in any other court of competent jurisdiction, of Indemnitee's entitlement to such indemnification or advancement of Expenses. Alternatively, Indemnitee, at Indemnitee's option, may seek an award in arbitration to be conducted by a single arbitrator pursuant to the Commercial Arbitration Rules of the American Arbitration Association. Indemnitee shall commence such proceeding seeking an adjudication within one hundred eighty (180) days following the date on which Indemnitee first has the right to commence such proceeding pursuant to this Section 7(a). The Company shall not oppose Indemnitee's right to seek any such adjudication or award in arbitration.

(b) In the event that a determination shall have been made pursuant to Section 6(b) of this Agreement that Indemnitee is not entitled to indemnification, any judicial proceeding commenced pursuant to this Section 7 shall be conducted in all respects as a de novo trial on the merits, and Indemnitee shall not be prejudiced by reason of the adverse determination under Section 6(b). In any judicial proceeding or arbitration commenced pursuant to this Section 7(b), the Company shall have the burden of proving Indemnitee is not entitled to indemnification or advancement of Expenses, as the case may be.

(c) If a determination shall have been made pursuant to Section 6(b) of this Agreement that Indemnitee is entitled to indemnification, the Company shall be bound by such determination in any judicial proceeding or arbitration commenced pursuant to this Section 7, absent (i) a misstatement by Indemnitee of a material fact, or an omission of a material fact necessary to make Indemnitee's statement not materially misleading in connection with the request for indemnification, or (ii) a prohibition of such indemnification under applicable law.

(d) In the event that Indemnitee, pursuant to this Section 7, seeks a judicial adjudication of such person's rights under, or to recover damages for breach of, this Agreement, or to recover under any directors' and officers' liability insurance policies maintained by the Company, the Company shall pay on Indemnitee's behalf, in advance, any and all expenses (of the types described in the definition of Expenses in Section 13 of this Agreement) actually and reasonably incurred by Indemnitee in such judicial adjudication, regardless of whether Indemnitee ultimately is determined to be entitled to such indemnification, advancement of expenses or insurance recovery.

(e) The Company, to the fullest extent not prohibited by law, shall be precluded from asserting in any judicial proceeding or arbitration commenced pursuant to this Section 7 that the procedures and presumptions of this Agreement are not valid, binding and enforceable and shall stipulate in any such court or before any such arbitrator that the Company is bound by all the provisions of this Agreement. It is the intent of the Company

that, to the fullest extent permitted by law, Indemnitee not be required to incur legal fees or other Expenses associated with the interpretation, enforcement or defense of Indemnitee's rights under this Agreement by litigation or otherwise because the cost and expense thereof would substantially detract from the benefits intended to be extended to Indemnitee hereunder. The Company shall, to the fullest extent permitted by law, indemnify Indemnitee against any and all Expenses and, if requested by Indemnitee, shall (within ten (10) days after receipt by the Company of a written request therefore) advance, to the extent not prohibited by law, such Expenses to Indemnitee, which are incurred by Indemnitee in connection with any action brought by Indemnitee for indemnification or advance of Expenses from the Company under this Agreement or under any directors' and officers' liability insurance policies maintained by the Company, regardless of whether Indemnitee ultimately is determined to be entitled to such indemnification, advancement of Expenses or insurance recovery, as the case may be.

(f) Notwithstanding anything in this Agreement to the contrary, no determination as to entitlement of Indemnitee to indemnification under this Agreement shall be required to be made prior to the final disposition of the Proceeding.

8. Non-Exclusivity; Survival of Rights; Insurance; Primacy of Indemnification; Subrogation.

(a) The rights of indemnification and to receive advancement of Expenses as provided by this Agreement shall not be deemed exclusive of any other rights to which Indemnitee may at any time be entitled under applicable law, the Fourth Amended and Restated Certificate of Incorporation of the Company (as amended and restated, from time to the "**Certificate of Incorporation**"), the Bylaws, any agreement, a vote of stockholders, a resolution of the Board or otherwise. No amendment, alteration or repeal of this Agreement or of any provision hereof shall limit or restrict any right of Indemnitee under this Agreement in respect of any action taken or omitted by such Indemnitee prior to such amendment, alteration or repeal. To the extent that a change in the DGCL, whether by statute or judicial decision, permits greater indemnification or advancement of Expenses than would be afforded currently under the Certificate of Incorporation, the Bylaws and this Agreement, it is the intent of the parties hereto that Indemnitee shall enjoy by this Agreement the greater benefits so afforded by such change. No right or remedy herein conferred is intended to be exclusive of any other right or remedy, and every other right and remedy shall be cumulative and in addition to every other right and remedy given hereunder or now or hereafter existing at law or in equity or otherwise. The assertion or employment of any right or remedy hereunder, or otherwise, shall not prevent the concurrent assertion or employment of any other right or remedy.

(b) To the extent that the Company maintains an insurance policy or policies providing liability insurance for certain directors, officers, employees, or agents or fiduciaries of the Company or of any other corporation, partnership, joint venture, trust, employee benefit plan or other enterprise that such person serves at the request of the Company, Indemnitee shall be covered by such policy or policies in accordance with its or their terms to the maximum extent of the coverage available for such director, officer, employee, agent or fiduciary under such policy or policies. If, at the time of the receipt of a notice of a claim pursuant to the terms hereof, the Company has directors' and officers' liability insurance in effect, the Company shall give prompt notice of such a claim or the commencement of a Proceeding, as the case may be, to the insurers in accordance with the procedures set forth in the respective policies. The Company shall thereafter take all necessary or desirable action to cause such insurers to pay, on behalf of Indemnitee, all amounts payable as a result of such Proceeding in accordance with the terms of such policies.

(c) The Company hereby acknowledges that Indemnitee has or may have certain rights to indemnification, advancement of expenses and/or insurance provided by other entities and/or organizations (collectively, the "**Fund Indemnitors**"). The Company hereby agrees (i) that it is the indemnitor of first resort (i.e., its obligations to Indemnitee are primary and any obligation of the Fund Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by Indemnitee are secondary), (ii) that it shall be required to advance the full amount of Expenses incurred by Indemnitee and shall be liable for the full amount of all Expenses, judgments, penalties, fines and amounts paid in settlement to the extent legally permitted and as required by the terms of this Agreement, the Certificate of Incorporation or the Bylaws (or any other agreement between the Company and Indemnitee), without regard to any rights Indemnitee may have against the Fund Indemnitors, and, (iii) that it irrevocably waives, relinquishes and releases the Fund Indemnitors from any and all claims against the Fund Indemnitors for contribution, subrogation or any other recovery of any kind in respect thereof. The Company further agrees that no advancement or payment by the Fund Indemnitors on behalf of Indemnitee with respect to any claim for which Indemnitee has sought indemnification from the Company shall affect the foregoing and the Fund Indemnitors shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to

all of the rights of recovery of Indemnitee against the Company. The Company and Indemnitee agree that the Fund Indemnitors are express third party beneficiaries of the terms of this Section 8(c).

(d) Except as provided in paragraph (c) above, in the event of any payment under this Agreement, the Company shall be subrogated to the extent of such payment to all of the rights of recovery of Indemnitee (other than against the Fund Indemnitors), who shall execute all papers required and take all action necessary to secure such rights, including execution of such documents as are necessary to enable the Company to bring suit to enforce such rights.

(e) Except as provided in paragraph (c) above, the Company shall not be liable under this Agreement to make any payment of amounts otherwise indemnifiable hereunder if and to the extent that Indemnitee has otherwise actually received such payment under any insurance policy, contract, agreement or otherwise.

(f) Except as provided in paragraph (c) above, the Company's obligation to indemnify or advance Expenses hereunder to Indemnitee who is or was serving at the request of the Company as a director, officer, employee or agent of any other corporation, partnership, joint venture, trust, employee benefit plan or other enterprise shall be reduced by any amount Indemnitee has actually received as indemnification or advancement of Expenses from such other corporation, partnership, joint venture, trust, employee benefit plan or other enterprise.

9. Exception to Right of Indemnification. Notwithstanding any provision in this Agreement, the Company shall not be obligated under this Agreement to make any indemnity in connection with any claim made against Indemnitee:

(a) for which payment has actually been made to or on behalf of Indemnitee under any insurance policy or other indemnity provision, except with respect to any excess beyond the amount paid under any insurance policy or other indemnity provision, provided, that the foregoing shall not affect the rights of Indemnitee or the Fund Indemnitors set forth in Section 8(c) above;

(b) for (i) an accounting of profits made from the purchase and sale (or sale and purchase) by Indemnitee of securities of the Company within the meaning of Section 16(b) of the Securities Exchange Act of 1934, as amended, or similar provisions of state statutory law or common law or (ii) any reimbursement of the Company by the Indemnitee of any bonus or other incentive-based or equity-based compensation or of any profits realized by the Indemnitee from the sale of securities of the Company, as required in each case under the Exchange Act (including any such reimbursements that arise from an accounting restatement of the Company pursuant to Section 304 of the Sarbanes-Oxley Act of 2002 (the "**Sarbanes-Oxley Act**"), or the payment to the Company of profits arising from the purchase and sale by Indemnitee of securities in violation of Section 306 of the Sarbanes-Oxley Act); or (iii) any reimbursement of the Company by Indemnitee of any compensation pursuant to any compensation recoupment or clawback policy adopted by the Board or the Compensation Committee of the Board, including but not limited to any such policy adopted to comply with stock exchange listing requirements implementing Section 10D of the Exchange Act; or

(c) in connection with any Proceeding (or any part of any Proceeding) initiated by Indemnitee, including any Proceeding (or any part of any Proceeding) initiated by Indemnitee against the Company or its directors, officers, employees or other indemnitees, unless (i) the Board authorized the Proceeding (or any part of any Proceeding) prior to its initiation or (ii) the Company provides the indemnification, in its sole discretion, pursuant to the powers vested in the Company under applicable law.

10. Duration of Agreement. All agreements and obligations of the Company contained herein shall continue during the period Indemnitee is an officer or director of the Company (or is or was serving at the request of the Company as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise) and shall continue thereafter so long as Indemnitee shall be subject to any Proceeding (or any proceeding commenced under Section 7 hereof) by reason of Indemnitee's Corporate Status, whether or not Indemnitee is acting or serving in any such capacity at the time any liability or expense is incurred for which indemnification can be provided under this Agreement. This Agreement shall be binding upon and inure to the benefit of and be enforceable by the parties hereto and their respective successors (including any direct or indirect successor by purchase, merger, consolidation or otherwise to all or substantially all of the business or assets of the Company), assigns, spouses, heirs, executors and personal and legal representatives.

Security. To the extent requested by Indemnitee and approved by the Board, the Company may at any time and from time to time provide security to Indemnitee for the Company's obligations hereunder through an irrevocable bank line of credit, funded trust or other collateral. Any such security, once provided to Indemnitee, may not be revoked or released without the prior written consent of Indemnitee.

11.

Enforcement. 12.

(a) The Company expressly confirms and agrees that it has entered into this Agreement and assumes the obligations imposed on it hereby in order to induce Indemnitee to serve as a director, an officer other person in service of the Company, and the Company acknowledges that Indemnitee is relying upon this Agreement in serving as a director, officer or other person in service of the Company.

(b) This Agreement constitutes the entire agreement between the parties hereto with respect to the subject matter hereof and supersedes all prior agreements and understandings, oral, written and implied, between the parties hereto with respect to the subject matter hereof, including any agreement covering the subject matter of this Agreement previously entered into between the Company and the Indemnitee; provided, however, that this Agreement is a supplement to and in furtherance of the Certificate of Incorporation, the Bylaws, and directors' and officers' insurance maintained by the Company and applicable law, and shall not be deemed a substitute therefor, nor to diminish or abrogate any rights of Indemnitee thereunder.

(c) The Company shall not seek from a court, or agree to, a "bar order" which would have the effect of prohibiting or limiting Indemnitee's rights to receive advancement of expenses under this Agreement.

Definitions. For purposes of this Agreement: 13.

(a) References to "agent" shall mean any person who is or was a director, officer, or employee of the Company or a subsidiary of the Company or other person authorized by the Company to act for the Company, to include such person serving in such capacity as a director, officer, employee, fiduciary or other official of another corporation, partnership, limited liability company, joint venture, trust or other enterprise at the request of, for the convenience of, or to represent the interests of the Company or a subsidiary of the Company.

(b) A "**Change in Control**" shall be deemed to occur upon the earliest to occur after the Effective Date of any of the following events:

(i) Acquisition of Stock by Third Party. Any Person (as defined below) is or becomes the Beneficial Owner (as defined below), directly or indirectly, of securities of the Company representing fifty percent (50%) or more of the combined voting power of the Company's then outstanding securities unless the change in relative Beneficial Ownership of the Company's securities by any Person results solely from a reduction in the aggregate number of outstanding shares of securities entitled to vote generally in the election of directors;

(ii) Change in Board of Directors. During any period of two (2) consecutive years (not including any period prior to the execution of this Agreement), individuals who at the beginning of such period constitute the Board, and any new director (other than a director designated by a person who has entered into an agreement with the Company to effect a transaction described in Sections 13(b)(i), 13(b)(iii) or 13(b)(iv)) whose election by the Board or nomination for election by the Company's stockholders was approved by a vote of at least two-thirds of the directors then still in office who either were directors at the beginning of the period or whose election or nomination for election was previously so approved, cease for any reason to constitute at least a majority of the members of the Board;

(ii) Corporate Transactions. The effective date of a merger or consolidation of the Company with any other entity, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior to such merger or consolidation continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or its ultimate parent, as applicable) more than fifty percent (50%) of the combined voting power of the voting securities of the surviving entity or its ultimate parent, as applicable, outstanding immediately after such merger or consolidation and with the power to elect at least a majority of the Board or other governing body of such surviving entity or its ultimate parent, as applicable;

(iii) Liquidation or Sale of Assets. The approval by the stockholders of the Company of a complete liquidation of the Company or an agreement for the sale or disposition by the Company of all or substantially all of the Company's assets; and

(iv) Other Events. There occurs any other event of a nature that would be required to be reported in response to Item 6(e) of Schedule 14A of Regulation 14A (or a response to any similar item on any similar schedule or form) promulgated under the Exchange Act (as defined below), whether or not the Company is then subject to such reporting requirement.

For purposes of this Section 13(b), the following terms shall have the following meanings:

(A) "Exchange Act" shall mean the Securities Exchange Act of 1934, as amended from time to time.

(B) "Person" shall have the meaning as set forth in Sections 13(d) and 14(d) of the Exchange Act; provided, however, that Person shall exclude (i) the Company, (ii) any trustee or other fiduciary holding securities under an employee benefit plan of the Company, and (iii) any entity owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their ownership of stock of the Company.

(C) "Beneficial Owner" shall have the meaning given to such term in Rule 13d-3 under the Exchange Act; provided, however, that Beneficial Owner shall exclude any Person otherwise becoming a Beneficial Owner by reason of the stockholders of the Company approving a merger of the Company with another entity.

(c) "**Corporate Status**" describes the status of a person who is or was a director, officer, employee, member, trustee or agent of the Company or of any other corporation, partnership, joint venture, trust, nonprofit entity or other enterprise (including service with respect to employee benefit plans) that such person is or was serving at the express written request of the Company.

(d) "**Disinterested Director**" means a director of the Company who is not and was not a party to the Proceeding in respect of which indemnification is sought by Indemnitee.

(e) "**Enterprise**" shall mean the Company and any other corporation, partnership, joint venture, trust, employee benefit plan or other enterprise that Indemnitee is or was serving at the express written request of the Company as a director, officer, employee, agent or fiduciary.

(f) "**Expenses**" shall include all reasonable attorneys' fees, retainers, court costs, transcript costs, fees of experts and other professionals, witness fees, travel expenses, duplicating costs, printing and binding costs, telephone charges, postage, delivery service fees, any federal, state, local or foreign taxes imposed on Indemnitee as a result of the actual or deemed receipt of any payments under this Agreement, ERISA excise taxes and penalties and all other disbursements or expenses of the types customarily incurred in connection with prosecuting, defending, preparing to prosecute or defend, investigating, participating, or being or preparing to be a witness in a Proceeding, or responding to, or objecting to, a request to provide discovery in any Proceeding. Expenses also shall include (i) Expenses incurred in connection with any appeal resulting from any Proceeding, including without limitation the premium, security for, and other costs relating to any cost bond, supersede as bond, or other appeal bond or its equivalent and (ii) Expenses incurred in connection with recovery under any directors' and officers' liability insurance policies maintained by the Company, regardless of whether Indemnitee is ultimately determined to be entitled to such indemnification, advancement, or Expenses or insurance recovery, as the case may be. The parties

agree that for the purposes of any advancement of Expenses for which Indemnitee has made written demand to the Company in accordance with this Agreement, all Expenses included in such demand that are certified by affidavit of Indemnitee's counsel as being reasonable in the good faith judgment of such counsel shall be presumed conclusively to be reasonable. Expenses, however, shall not include amounts paid in settlement by Indemnitee or the amount of judgments or fines against Indemnitee.

(g) "**Independent Counsel**" means a law firm, or a member of a law firm, that is experienced in matters of corporation law and neither presently is, nor in the past five years has been, retained to represent: (i) the Company or Indemnitee in any matter material to either such party (other than with respect to matters concerning Indemnitee under this Agreement, or of other indemnitees under similar indemnification agreements), or (ii) any other party to the Proceeding giving rise to a claim for indemnification hereunder. Notwithstanding the foregoing, the term "Independent Counsel" shall not include any person who, under the applicable standards of professional conduct then prevailing, would have a conflict of interest in representing either the Company or Indemnitee in an action to determine Indemnitee's rights under this Agreement. The Company agrees to pay the reasonable fees and expenses of the Independent Counsel referred to above and to fully indemnify such counsel against any and all Expenses, claims, liabilities and damages arising out of or relating to this Agreement or its engagement pursuant hereto.

(h) "**Proceeding**" shall include any threatened, pending or completed action, suit, claim, counterclaim, cross claim, arbitration, mediation, alternate dispute resolution mechanism, investigation, inquiry, administrative hearing or any other actual, threatened or completed proceeding, whether brought by or in the right of the Company or otherwise and whether civil, criminal, administrative, regulatory or investigative, in which Indemnitee was, is or will be involved as a party, potential party, non-party witness or otherwise, by reason of Indemnitee's Corporate Status, by reason of any action taken by Indemnitee or of any inaction on Indemnitee's part while acting in Indemnitee's Corporate Status; in each case whether or not Indemnitee is acting or serving in any such capacity at the time any liability or expense is incurred for which indemnification, reimbursement, or advancement of Expenses can be provided under this Agreement; including one pending on or before the date of this Agreement, but excluding one initiated by an Indemnitee pursuant to Section 7 of this Agreement to enforce Indemnitee's rights under this Agreement.

(i) Reference to "other enterprise" shall include employee benefit plans; references to "fines" shall include any excise tax assessed with respect to any employee benefit plan; references to "serving at the request of the Company" shall include any service as a director, officer, employee or agent of the Company which imposes duties on, or involves services by, such director, officer, employee or agent with respect to an employee benefit plan, its participants or beneficiaries; and a person who acted in good faith and in a manner Indemnitee reasonably believed to be in the best interests of the participants and beneficiaries of an employee benefit plan shall be deemed to have acted in a manner "not opposed to the best interests of the Company" as referred to in this Agreement.

Partial Indemnification. 14. If Indemnitee is entitled under any provision of this Agreement to indemnification by the Company for some or a portion of Expenses, but not, however, for the total amount thereof, the Company shall nevertheless indemnify Indemnitee for the portion thereof to which Indemnitee is entitled.

Severability. 15. The invalidity or unenforceability of any provision hereof shall in no way affect the validity or enforceability of any other provision. Further, the invalidity or unenforceability of any provision hereof as to either Indemnitee shall in no way affect the validity or enforceability of any provision hereof as to the other. Without limiting the generality of the foregoing, this Agreement is intended to confer upon Indemnitee indemnification rights to the fullest extent permitted by applicable laws. In the event any provision hereof conflicts with any applicable law, such provision shall be deemed modified, consistent with the aforementioned intent, to the extent necessary to resolve such conflict.

Modification and Waiver. 16. No supplement, modification, termination or amendment of this Agreement shall be binding unless executed in writing by both of the parties hereto. No waiver of any of the provisions of this Agreement shall be deemed or shall constitute a waiver of any other provisions hereof (whether or not similar) nor shall such waiver constitute a continuing waiver.

Notice By Indemnitee. 17. Indemnitee agrees promptly to notify the Company in writing upon being served with or otherwise receiving any summons, citation, subpoena, complaint, indictment, information or

other document relating to any Proceeding or matter which may be subject to indemnification covered hereunder. The failure to so notify the Company shall not relieve the Company of any obligation which it may have to Indemnitee under this Agreement or otherwise unless and only to the extent that such failure or delay materially prejudices the Company.

18. Notices. All notices and other communications given or made pursuant to this Agreement shall be in writing and shall be deemed effectively given: (a) upon personal delivery to the party to be notified, (b) when sent by confirmed electronic mail or facsimile if sent during normal business hours of the recipient, and if not so confirmed, then on the next business day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid, or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications shall be sent:

(a) To Indemnitee at the address set forth below Indemnitee signature hereto.

(b) To the Company at:

LogicBio Therapeutics, Inc.
65 Hayden Avenue, Floor 2
Lexington, MA 02421
Attention: General Counsel
Email: legal@logicbio.com

or to such other address as may have been furnished to Indemnitee by the Company or to the Company by Indemnitee, as the case may be.

19. Counterparts. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same Agreement. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature complying with the U.S. federal ESIGN Act of 2000, e.g., www.docusign.com) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes.

20. Headings. The headings of the paragraphs of this Agreement are inserted for convenience only and shall not be deemed to constitute part of this Agreement or to affect the construction thereof.

21. Governing Law and Consent to Jurisdiction. This Agreement and the legal relations between the parties hereto shall be governed by, and construed and enforced in accordance with, the laws of the State of Delaware, without regard to its conflict of laws rules. The Company and Indemnitee hereby irrevocably and unconditionally (i) agree that any action or proceeding arising out of or in connection with this Agreement shall be brought only in the Delaware Court, and not in any other state or federal court in the United States of America or any court in any other country, (ii) consent to submit to the exclusive jurisdiction of the Delaware Court for purposes of any action or proceeding arising out of or in connection with this Agreement, (iii) waive any objection to the laying of venue of any such action or proceeding in the Delaware Court, and (iv) waive, and agree not to plead or to make, any claim that any such action or proceeding brought in the Delaware Court has been brought in an improper or inconvenient forum.

SIGNATURE PAGE TO FOLLOW

IN WITNESS WHEREOF, the parties hereto have executed this Indemnification Agreement on and as of the day and year first above written.

LOGICBIO THERAPEUTICS, INC.

By:_____
Name:
Title:

INDEMNITEE

_____
Name:
Address:

Exhibit 10.2

Name:	[]
Number of Shares of Stock subject to the Stock Option:	[]
Exercise Price Per Share:	$[]
Date of Grant:	[]
Vesting Commencement Date:	[]

LOGICBIO THERAPEUTICS, INC.
INDUCEMENT AWARD AGREEMENT

This agreement (this "**Agreement**") evidences an inducement award granted by LogicBio Therapeutics, Inc. (the "**Company**") to the individual named above (the "**Participant**"), consisting of an option to purchase shares of Stock. Except as otherwise defined herein, all capitalized terms used herein have the same meaning as in the LogicBio Therapeutics, Inc. 2018 Equity Incentive Plan (as from time to time amended and in effect, the "**Plan**").

1. **Grant of Stock Option.** The Company grants to the Participant on the date set forth above (the "**Date of Grant**") an option (the "**Stock Option**") to purchase up to the number of shares of Stock set forth above (the "**Shares**"), with an exercise price per Share as set forth above, in each case subject to adjustment pursuant to Section 7 of the Plan in respect of transactions occurring after the date hereof. The option is to be granted to the Participant in connection with the Participant entering into employment with the Company as an inducement material to the Participant's entering into employment with the Company within the meaning of Nasdaq Listing Rule 5635(c)(4).

The Stock Option evidenced by this Agreement is a non-statutory option (that is, an option that is not intended to qualify as an ISO) and is granted to the Participant in connection with the Participant's Employment.

2. **Relationship to and Incorporation of the Plan.** The Stock Option shall be subject to and governed by, and shall be construed and administered in accordance with, the terms and conditions of the Plan, as amended from time to time, which terms and conditions are incorporated herein by reference. A copy of the Plan has been made available to the Participant. Notwithstanding the foregoing, the Stock Option is not awarded under the Plan and the grant of the Stock Option and issuance of any Shares pursuant to the exercise of the Stock Option shall not reduce the number of shares of Stock available for issuance under awards issued pursuant to the Plan. By accepting the Stock Option, the Participant agrees to be bound by the terms and conditions set forth in this Agreement.

3. **Vesting.** The term "**vest**" as used herein with respect to the Stock Option (or any portion thereof) means to become exercisable and the term "**vested**" with respect to the Stock Option (or any portion) means that the Stock Option (or portion) is then exercisable. Unless earlier terminated, forfeited, relinquished or expired, the Stock Option will vest [ ].

4. **Exercise of the Stock Option.** No portion of the Stock Option may be exercised until such portion vests. Each election to exercise any vested portion of the Stock Option will be subject to the terms and conditions of the Plan and must be in written or electronic form acceptable to the Administrator, signed (including by electronic signature) by the Participant or, if at the relevant time the Stock Option has passed to a Beneficiary or permitted transferee, the Beneficiary or permitted transferee. Each such written or electronic exercise election must be received by the Company at its principal office or by such other party as the Administrator may prescribe and be accompanied by payment in full of the exercise price as provided in the Plan. The latest date on which the Stock Option or any portion thereof may be exercised is the tenth (10th) anniversary of the Date of Grant (the "**Final Exercise Date**") and, if

not exercised by such date, the Stock Option or any remaining portion thereof will thereupon immediately terminate.

5. **Cessation of Employment.** If the Participant's Employment ceases, except as expressly provided for in an employment, severance, separation or other similar agreement between the Participant and the Company that is in effect at the time of such termination, the Stock Option, to the extent not then vested, will be immediately forfeited for no consideration, and any vested portion of the Stock Option that is then outstanding will remain exercisable for the period described in Section 6(a)(4) of the Plan.

6. **Restrictions on Transfer.** The Stock Option may not be transferred except as expressly permitted under Section 6(a)(3) of the Plan.

7. **Forfeiture; Recovery of Compensation.** By accepting the Stock Option, the Participant expressly acknowledges and agrees that the Participant's rights, and those of any permitted transferee, with respect to the Stock Option, including the right to any Shares acquired under the Stock Option or proceeds from the disposition thereof, are subject to Section 6(a)(5) of the Plan (including any successor provision). Nothing in the preceding sentence will be construed as limiting the general application of Section 2 of this Agreement.

8. **Withholding.** The Participant expressly acknowledges and agrees that the Participant's rights hereunder, including the right to be issued Shares upon exercise of the Stock Option, are subject to the Participant promptly paying to the Company in cash or by check (or by such other means as may be acceptable to the Administrator) all taxes required to be withheld, if any. No Shares will be issued pursuant to the exercise of the Stock Option unless and until the person exercising the Stock Option has remitted to the Company an amount in cash sufficient to satisfy any federal, state, or local withholding tax requirements, or has made other arrangements satisfactory to the Company with respect to such taxes. The Participant authorizes the Company and its subsidiaries to withhold such amount from any amounts otherwise owed to the Participant, but nothing in this sentence will be construed as relieving the Participant of any liability for satisfying his or her obligation under the preceding provisions of this Section 8.

9. **Acknowledgements.** The Participant acknowledges and agrees that (i) this Agreement may be executed in two or more counterparts, each of which will be an original and all of which together will constitute one and the same instrument; (ii) this Agreement may be executed and exchanged using facsimile, portable document format (PDF) or electronic signature, which, in each case, will constitute an original signature for all purposes hereunder; and (iii) such signature by the Company will be binding against the Company and will create a legally binding agreement when this Agreement is countersigned by the Participant.

[*Signature page follows.*]

The Company, by its duly authorized officer, and the Participant have executed this Agreement as of the Date of Grant.

LogicBio Therapeutics, Inc.

By:
Name:
Title:

Agreed and Accepted:

By:
[Participant's Name]

[*Signature Page to Inducement Award Agreement*]

Exhibit 31.1

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

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

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

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

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

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

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

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

Date: November 15, 2021

By: /s/ Frederic Chereau
Frederic Chereau
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

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, Cecilia Jones, 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 and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

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

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

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

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

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

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

Date: November 15, 2021

By: /s/ Cecilia Jones
Cecilia Jones
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of LogicBio Therapeutics, Inc. (the "Company") for the quarter ended September 30, 2021 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 or her knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 15, 2021

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

Dated: November 15, 2021

/s/ Cecilia Jones
Cecilia Jones
Chief Financial Officer