

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 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: 001-38210

**Krystal Biotech, Inc.**  
(Exact name of registrant as specified in its charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

82-1080209  
(I.R.S. Employer  
Identification Number)

2100 Wharton Street, Suite 701  
Pittsburgh, Pennsylvania 15203  
(Address of principal executive offices and zip code)  
(412) 586-5830  
(Registrant's telephone number, including area code)

N/A  
(Former name, former address and former 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	KRYS	NASDAQ

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

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

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

Large accelerated filer  Accelerated filer   
Non-accelerated filer  Smaller reporting company

Emerging growth company

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

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

As of October 29, 2021, there were 22,237,984 shares of the registrant's common stock issued and outstanding.

**Krystal Biotech, Inc.**  
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**PART I. FINANCIAL INFORMATION**  
**ITEM 1. FINANCIAL STATEMENTS (UNAUDITED)**

**Krystal Biotech, Inc.**  
**Condensed Consolidated Balance Sheets**

<b>(In thousands, except shares and per share data)</b>	<b>(unaudited)</b> <b>September 30,</b> <b>2021</b>	<b>December 31,</b> <b>2020</b>
<b>Assets</b>		
Current assets		
Cash and cash equivalents	\$ 286,614	\$ 268,269
Short-term investments	56,479	2,993
Prepaid expenses and other current assets	2,719	3,796
Total current assets	345,812	275,058
Property and equipment, net	78,642	30,876
Long-term investments	19,237	—
Right-of-use assets	7,342	3,298
Other non-current assets	62	1,612
Total assets	<u>\$ 451,095</u>	<u>\$ 310,844</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities		
Accounts payable	\$ 4,757	\$ 2,105
Current portion of lease liability	1,023	638
Accrued expenses and other current liabilities	29,999	5,109
Build to suit lease liability	—	7,600
Total current liabilities	35,779	15,452
Lease liability	7,079	3,308
Total liabilities	42,858	18,760
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred stock; \$0.00001 par value; 20,000,000 shares authorized at September 30, 2021 (unaudited) and December 31, 2020; 2,061,773 shares issued, and no shares outstanding at September 30, 2021 (unaudited) and December 31, 2020	—	—
Common stock; \$0.00001 par value; 80,000,000 shares authorized at September 30, 2021 (unaudited) and December 31, 2020; 22,237,984 and 19,714,220 shares issued and outstanding at September 30, 2021 (unaudited) and December 31, 2020, respectively	—	—
Additional paid-in capital	527,229	363,292
Accumulated other comprehensive income (expense)	(14)	6
Accumulated deficit	(118,978)	(71,214)
Total stockholders' equity	408,237	292,084
Total liabilities and stockholders' equity	<u>\$ 451,095</u>	<u>\$ 310,844</u>

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

**Krystal Biotech, Inc.**  
**Condensed Consolidated Statements of Operations and Comprehensive Loss**  
**(unaudited)**

(In thousands, except share and per share data)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
<b>Expenses</b>				
Research and development	\$ 6,080	\$ 5,100	\$ 18,875	\$ 12,264
General and administrative	9,572	4,580	27,524	10,315
Total operating expenses	15,652	9,680	46,399	22,579
Loss from operations	(15,652)	(9,680)	(46,399)	(22,579)
<b>Other Income (Expense)</b>				
Interest and other income, net	63	70	127	795
Interest expense	—	—	(1,492)	—
Net loss	(15,589)	(9,610)	(47,764)	(21,784)
Unrealized gain (loss) on available-for-sale securities	7	(20)	(20)	10
Comprehensive loss	\$ (15,582)	\$ (9,630)	\$ (47,784)	\$ (21,774)
Net loss per common share:				
Basic and diluted	\$ (0.70)	\$ (0.49)	\$ (2.18)	\$ (1.18)
Weighted-average common shares outstanding:				
Basic and diluted	22,212,266	19,676,016	21,893,656	18,477,495

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

**Krystal Biotech, Inc.**  
**Condensed Consolidated Statements of Stockholders' Equity**  
**(unaudited)**

(In thousands, except shares)	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
<b>Balances at January 1, 2021</b>	19,714,220	\$ —	\$ 363,292	\$ 6	\$ (71,214)	\$ 292,084
Issuance of common stock, net	2,489,837	—	152,033	—	—	152,033
Stock-based compensation expense	—	—	2,350	—	—	2,350
Unrealized loss on investments	—	—	—	(3)	—	(3)
Net loss	—	—	—	—	(15,812)	(15,812)
<b>Balances at March 31, 2021</b>	22,204,057	\$ —	\$ 517,675	\$ 3	\$ (87,026)	\$ 430,652
Issuance of common stock, net	975	—	14	—	—	14
Stock-based compensation expense	—	—	4,261	—	—	4,261
Unrealized loss on investments	—	—	—	(24)	—	(24)
Net loss	—	—	—	—	(16,363)	(16,363)
<b>Balances at June 30, 2021</b>	22,205,032	\$ —	\$ 521,950	\$ (21)	\$ (103,389)	\$ 418,540
Issuance of common stock, net	32,952	—	1,534	—	—	1,534
Stock-based compensation expense	—	—	3,745	—	—	3,745
Unrealized gain on investments	—	—	—	7	—	7
Net loss	—	—	—	—	(15,589)	(15,589)
<b>Balances at September 30, 2021</b>	22,237,984	\$ —	\$ 527,229	\$ (14)	\$ (118,978)	\$ 408,237

(In thousands, except shares)	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
<b>Balances at January 1, 2020</b>	17,354,310	\$ —	\$ 241,951	\$ 10	\$ (39,047)	\$ 202,914
Issuance of common stock, net	16,254	—	243	—	—	243
Stock-based compensation expense	—	—	539	—	—	539
Unrealized gain on investments	—	—	—	14	—	14
Net loss	—	—	—	—	(5,341)	(5,341)
<b>Balances at March 31, 2020</b>	17,370,564	\$ —	\$ 242,733	\$ 24	\$ (44,388)	\$ 198,369
Issuance of common stock, net	2,293,495	—	117,337	—	—	117,337
Stock-based compensation expense	—	—	807	—	—	807
Unrealized gain on investments	—	—	—	16	—	16
Net loss	—	—	—	—	(6,833)	(6,833)
<b>Balances at June 30, 2020</b>	19,664,059	\$ —	\$ 360,877	\$ 40	\$ (51,221)	\$ 309,696
Issuance of common stock, net	42,811	—	298	—	—	298
Stock-based compensation expense	—	—	1,356	—	—	1,356
Unrealized loss on investments	—	—	—	(20)	—	(20)
Net loss	—	—	—	—	(9,610)	(9,610)
<b>Balances at September 30, 2020</b>	19,706,870	\$ —	\$ 362,531	\$ 20	\$ (60,831)	\$ 301,720

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

**Krystal Biotech, Inc.**  
**Condensed Consolidated Statements of Cash Flows**  
**(unaudited)**

(In thousands)	Nine Months Ended September 30,	
	2021	2020
<b>Operating Activities</b>		
Net loss	\$ (47,764)	\$ (21,784)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation and amortization	1,985	1,361
Stock-based compensation expense	10,174	2,702
Loss on disposals of fixed assets	—	33
Non-cash interest expense	1,492	—
Changes in operating assets and liabilities		
Prepaid expenses and other current assets	814	489
Prepaid rent	—	(2,400)
Lease liability	(240)	(165)
Accounts payable	(225)	725
Accrued expenses and other current liabilities	6,726	980
Net cash used in operating activities	(27,038)	(18,059)
<b>Investing Activities</b>		
Purchases of property and equipment	(27,453)	(7,636)
Purchases of investments	(83,810)	(3,205)
Proceeds from maturities of investments	11,033	5,877
Net cash used in investing activities	(100,230)	(4,964)
<b>Financing Activities</b>		
Issuance of common stock, net	153,573	117,878
Repayment of ASTRA build to suit liability	(7,960)	—
Net cash provided by financing activities	145,613	117,878
Net increase in cash and cash equivalents	18,345	94,855
Cash and cash equivalents at beginning of period	268,269	187,514
Cash and cash equivalents at end of period	\$ 286,614	\$ 282,369
<b>Supplemental Disclosures of Non-Cash Investing and Financing Activities</b>		
Unpaid purchases of property and equipment	\$ 23,213	\$ 3,173
Unpaid offering costs	\$ 11	\$ —
Initial recognition of right-of-use assets and modification	\$ 4,396	\$ —

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

**Krystal Biotech, Inc.**  
**Notes to Condensed Consolidated Financial Statements**  
**(unaudited)**

**1. Organization**

Krystal Biotech, Inc. (the “Company,” or “we” or other similar pronouns) commenced operations on April 15, 2016. On March 31, 2017, the Company converted from a California limited liability company to a Delaware C-corporation, and changed its name from Krystal Biotech LLC to Krystal Biotech, Inc. On June 19, 2018, the Company incorporated Krystal Australia Pty Ltd., an Australian proprietary limited company, a wholly-owned subsidiary, for the purpose of undertaking preclinical and clinical studies in Australia. On April 24, 2019, the Company incorporated Jeune Aesthetics, Inc., formerly known as Jeune, Inc. (“Jeune”), in Delaware, a wholly-owned subsidiary, for the purpose of undertaking preclinical and clinical studies for aesthetic skin conditions.

We are a clinical stage biotechnology company leading the field of redosable gene delivery for the treatment of serious rare diseases. Using our patented platform that is based on engineered herpes simplex virus type 1 (“HSV-1”), we create vectors that efficiently deliver therapeutic transgenes to cells of interest in multiple organ systems. The cell’s own machinery then transcribes and translates the encoded effector to treat or prevent disease. We formulate our vectors for non-invasive or minimally invasive routes of administration at a doctor’s office or potentially in the patient’s home by a healthcare professional. Our goal is to develop easy to use, medicines to dramatically improve the lives of patients living with rare diseases. Our innovative technology platform is supported by in-house, commercial scale current good manufacturing practices (“cGMP”) manufacturing capabilities.

**Liquidity**

As of September 30, 2021, the Company had an accumulated deficit of \$119.0 million. With the net proceeds raised from its public and private securities offerings, including the public offering of its common stock completed on February 1, 2021, the Company believes that its cash, cash equivalents and short-term investments of approximately \$343.1 million as of September 30, 2021 will be sufficient to allow the Company to fund its planned operations for at least the next 12 months from the date of this Quarterly Report on Form 10-Q. As the Company continues to incur losses, a transition to profitability is dependent upon the successful development, approval and commercialization of its product candidates and the achievement of a level of revenues adequate to support the Company’s cost structure. The Company may never achieve profitability and unless and until it does, the Company will continue to need to raise additional capital or obtain financing from other sources. Management intends to fund future operations through the sale of equity and debt financings and may also seek additional capital through arrangements with strategic partners or other sources. There can be no assurance that additional funding will be available on terms acceptable to the Company, if at all.

The Company is subject to risks common to companies in the biotechnology industry, including but not limited to the failure of product candidates in clinical and preclinical studies, the development of competing product candidates or other technological innovations by competitors, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to commercialize product candidates.

**2. Summary of Significant Accounting Policies**

**Basis of Presentation**

The accompanying unaudited interim condensed financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America (“GAAP”), as found in the Accounting Standards Codification (“ASC”), the Accounting Standards Update (“ASU”), of the Financial Accounting Standards Board (“FASB”), and the rules and regulations of the US Securities and Exchange Commission (“SEC”). In the opinion of management, all adjustments, which consist of all normal recurring adjustments necessary for a fair presentation of the Company’s financial position and results of operations for the interim periods ended September 30, 2021 and 2020, are reflected in the interim condensed consolidated financial statements. All intercompany balances and transactions have been eliminated in consolidation.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These unaudited interim condensed 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 fiscal year ended December 31, 2020, as filed with the SEC on March 1, 2021.

### ***Risks and Uncertainties***

The novel coronavirus ("COVID-19") pandemic has resulted, and is likely to continue to result, in significant national and global economic uncertainty and may adversely affect our business. The Company is continuing to actively monitor the impact of the COVID-19 pandemic and the related effects on its financial condition, liquidity, operations, suppliers, industry, and workforce. However, the full extent, consequences, and duration of the COVID-19 pandemic and the resulting impact on the Company cannot currently be predicted. The Company will continue to evaluate the impact that these events could have on the operations, financial position, and the results of operations and cash flows during fiscal year 2021.

### ***Use of Estimates***

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the condensed consolidated financial statements and accompanying notes. Actual results could materially differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the financial statements. Estimates are used in the following areas including stock-based compensation expense, accrued expenses, the fair value of financial instruments, the incremental borrowing rate for lease liabilities, and the valuation allowance included in the deferred income tax calculation.

### ***Segment and Geographical Information***

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company and the Company's chief operating decision maker view the Company's operations and manage its business in one operating segment, which is the business of developing and commercializing pharmaceuticals.

### ***Concentrations of Credit Risk and Off-Balance Sheet Risk***

Financial instruments that potentially subject the Company to credit risk consist of cash, cash equivalents and investments. The Company's policy is to invest its cash, cash equivalents and investments in money market funds, certificates of deposit, corporate bonds, commercial paper, government agency securities and various other bank deposit accounts. The counterparties to the agreements relating to the Company's investments consist of financial institutions of high credit standing. The Company is exposed to credit risk in the event of default by the financial institutions to the extent amounts recorded on the balance sheets are in excess of insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds. The Company has no financial instruments with off-balance sheet risk of loss.

### ***Cash, Cash Equivalents and Investments***

Cash and cash equivalents consist of money market funds and bank deposits. Cash equivalents are defined as short-term, highly liquid investments with original maturities of 90 days or less at the date of purchase.

Investments with maturities of greater than 90 days but less than one year are classified as short-term investments on the consolidated balance sheets and consist of certificates of deposit, commercial paper, corporate bonds, and government agency securities. Investments with maturities of greater than one year are classified as long-term investments on the consolidated balance sheets and consist of corporate bonds and government agency securities. Accrued interest on corporate bonds and government agency securities are also classified as short-term investments.

As our entire investment portfolio is considered available for use in current operations, we classify all investments as available-for-sale securities. Available-for-sale securities are carried at fair value, with unrealized gains and losses reported in accumulated other comprehensive loss, which is a separate component of stockholders' equity in the consolidated balance sheets.



### ***Fair Value of Financial Instruments***

Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. There is a three-level hierarchy that prioritizes the inputs used in determining fair value by their reliability and preferred use, as follows:

- *Level 1*— Valuations based on quoted prices in active markets for identical assets or liabilities.
- *Level 2*— Valuations based on quoted prices in active markets for similar assets and liabilities, quoted prices for identical or similar assets or liabilities in inactive markets, or other inputs that are observable or can be corroborated by observable market data.
- *Level 3*— Valuations based on inputs that are both significant to the fair value measurement and unobservable.

To the extent that a valuation is based on models or inputs that are less observable, or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized within Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

There have been no significant changes to the valuation methods utilized by the Company during the periods presented. There have been no transfers between Level 1, Level 2, and Level 3 in any periods presented.

The carrying amounts of financial instruments consisting of cash and cash equivalents, investments, prepaid expenses and other current assets, accounts payable, accrued expenses and other current liabilities included in the Company's financial statements, are reasonable estimates of fair value, primarily due to their short maturities. Marketable securities are classified as long-term investments if the Company has the ability and intent to hold them and such holding period is longer than one year. The Company classifies all of its investments as available-for-sale.

Our available-for-sale, short-term and long-term investments, which consist of certificates of deposit, commercial paper, corporate bonds, and government agency securities are considered to be Level 2 valuations. The fair value of Level 2 financial assets is determined using inputs that are observable in the market or can be derived principally from or corroborated by observable market data, such as pricing for similar securities, recently executed transactions, cash flow models with yield curves, and benchmark securities. In addition, Level 2 financial instruments are valued using comparisons to like-kind financial instruments and models that use readily observable market data as their basis.

### ***Property and Equipment, net***

Property and equipment, net, is stated at cost, less accumulated depreciation. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred, while costs of major additions and betterments are capitalized. Upon disposal, the related cost and accumulated depreciation is removed from the accounts and any resulting gain or loss is included in the results of operations. Depreciation is recorded using the straight-line method over the estimated useful lives of the respective assets, which are as follows:

Computer equipment and software	3 years
Lab equipment	3 -7 years
Furniture and fixtures	3 years
Leasehold improvement	lesser of remaining useful life or remaining lease term

Construction in progress ("CIP") is not depreciated until the asset is placed in service.

### ***Impairment of Long-Lived Assets***

The Company evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. An impairment loss would be recognized when estimated future cash flows expected to result from the use of the asset and its eventual disposition are less than the carrying amount of the asset. The Company has not recognized any impairment losses for the three and nine months ended September 30, 2021 and 2020.

## **Leases**

The Company accounts for its lease agreements in accordance with FASB ASC Topic 842, Leases ("ASC 842"). Right-of-use lease assets represent our right to use an underlying asset during the lease term and the lease obligations represent our commitment to make lease payments arising from the lease. Right-of-use lease assets and obligations are recognized based on the present value of remaining lease payments over the lease term. As the Company's lease agreements do not provide an implicit rate and as the Company does not have any external borrowings, we have used an estimated incremental borrowing rate based on the information available at lease commencement in determining the present value of lease payments. Operating lease expense is recognized on a straight-line basis over the lease term. Variable lease expense is recognized in the period in which the obligation for the payment is incurred. In addition, the Company also has made an accounting policy election to exclude leases with an initial term of twelve months or less from its balance sheet and to account for lease and non-lease components of its operating leases as a single component.

For lease arrangements where it has been determined that the Company has control over an asset that is under construction and is thus considered the accounting owner of the asset during the construction period, the Company records a construction in progress asset and corresponding financial obligation on the condensed consolidated balance sheet. Once the construction is complete, an assessment is performed to determine whether the lease meets certain "sale-leaseback" criteria. If the sale-leaseback criteria are determined to be met, the Company will remove the asset and related financial obligation from the condensed consolidated balance sheet and treat the lease as either an operating or finance lease based on our assessment of the guidance. If, upon completion of construction, the project does not meet the "sale-leaseback" criteria, the lease will be treated as a financing obligation and the Company will depreciate the asset over its estimated useful life for financial reporting purposes once the asset has been placed into service.

## **Research and Development Expenses**

Research and development costs are charged to expense as incurred in performing research and development activities. These costs include employee compensation costs, facilities and overhead, preclinical and clinical activities, related clinical manufacturing costs, contract management services, regulatory and other related costs.

The Company estimates contract research and clinical trials materials manufacturing expenses based on the services performed pursuant to contracts with research organizations and manufacturing organizations that manufacture materials used in the Company's ongoing preclinical and clinical studies. Non-refundable advanced payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. These estimates are based on communications with the third party service providers and the Company's estimates of accrued expenses using information available at each balance sheet date. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company will adjust the accrual accordingly.

## **Stock-Based Compensation Expense**

The Company accounts for its stock-based compensation awards in accordance with FASB ASC Topic 718, Compensation-Stock Compensation ("ASC 718"). ASC 718 requires all stock-based payments, including grants of stock options and restricted stock, to be recognized in the statements of operations based on their grant-date fair values. Compensation expense is recognized on a straight-line basis based on the grant-date fair value over the associated service period of the award, which is generally the vesting term.

The Company estimates the fair value of its stock options using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including: (i) the expected stock price volatility; (ii) the expected term of the award; (iii) the risk-free interest rate; and (iv) expected dividends. Due to the lack of sufficient history and trading volume of our Common Stock and a lack of Company-specific historical and implied volatility data, the Company has based its estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. When selecting these public companies on which it has based its expected stock price volatility, the Company selected companies with comparable characteristics to it, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected term of the stock-based awards. The Company computes historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.

Due to the lack of Company-specific historical option activity, the Company has estimated the expected term of its employee and non-employee stock options using the “simplified” method, whereby the expected term equals the arithmetic mean of the vesting term and the original contractual term of the option. The risk-free interest rates are based on the US Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid and does not expect to pay dividends in the foreseeable future. The Company accounts for forfeitures as they occur. Stock-based compensation expense recognized in the financial statements is based on awards for which service conditions are expected to be satisfied.

### Comprehensive Loss

Comprehensive loss is defined as the change in equity during a period from transactions from non-owner sources. Unrealized gains or losses on available-for-sale securities is a component of other comprehensive gains or losses and is presented net of taxes. We have not recorded any reclassifications from other comprehensive gains or losses to net loss during any period presented.

### Recent Accounting Pronouncements

#### ASU No. 2020-08, Codification Improvements to Subtopic 310-20, Receivables - Nonrefundable Fees and Other Costs

In October 2020, the FASB issued ASU 2020-08, *Codification Improvements to Subtopic 310-20, Receivables - Nonrefundable Fees and Other Costs* (“ASU 2020-08”) to provide further clarification and update the previously issued guidance in ASU 2017-08, *Receivables - Nonrefundable Fees and Other Costs (Subtopic 310-20: Premium Amortization on Purchased Callable Debt Securities)* (“ASU 2017-08”). ASU 2017-08 shortened the amortization period for certain callable debt securities purchased at a premium by requiring that the premium be amortized to the earliest call date. ASU 2020-08 requires that at each reporting period, to the extent that the amortized cost of an individual callable debt security exceeds the amount repayable by the issuer at the next call date, the excess premium shall be amortized to the next call date. The new standard was effective beginning January 1, 2021 and should be applied on a prospective basis as of the beginning of the period of adoption for existing or newly purchased callable debt securities. The adoption of ASU 2020-08 did not have a material impact on the Company’s financial position or results of operations upon adoption.

### 3. Net Loss Per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing net loss attributable to common stockholders by the weighted average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share attributable to common stockholders is computed by dividing the net loss by the weighted-average number of shares of common stock and common share equivalents outstanding for the period. Common share equivalents consist of common stock issuable upon exercise of stock options and vesting of restricted stock awards. There were 1,900,638 and 853,336 common share equivalents outstanding as of September 30, 2021 and 2020, respectively, in the form of stock options and unvested restricted stock awards, that have been excluded from the calculation of diluted net loss per common share as their effect would be anti-dilutive for all periods presented.

(In thousands, except shares and per share data)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
	(Unaudited)		(Unaudited)	
<b>Numerator:</b>				
Net loss per common share	\$ (15,589)	\$ (9,610)	\$ (47,764)	\$ (21,784)
<b>Denominator:</b>				
Weighted-average basic and diluted common shares	22,212,266	19,676,016	21,893,656	18,477,495
Basic and diluted net loss per common share	\$ (0.70)	\$ (0.49)	\$ (2.18)	\$ (1.18)

#### 4. Fair Value Instruments

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of September 30, 2021 and December 31, 2020, respectively (in thousands):

	September 30, 2021 (unaudited)						
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities <sup>(1)</sup>	Long-term Marketable Securities <sup>(2)</sup>
<b>Level 1:</b>							
Cash and cash equivalents	\$ 286,614	\$ —	\$ —	\$ 286,614	\$ 286,614	\$ —	\$ —
Subtotal	286,614	—	—	286,614	286,614	—	—
<b>Level 2:</b>							
Commercial paper	22,490	2	—	22,492	—	22,492	—
Corporate bonds	37,683	1	(26)	37,658	—	29,465	8,193
U.S. government agency securities	15,567	2	(3)	15,566	—	4,522	11,044
Subtotal	75,740	5	(29)	75,716	—	56,479	19,237
<b>Total</b>	<b>\$ 362,354</b>	<b>\$ 5</b>	<b>\$ (29)</b>	<b>\$ 362,330</b>	<b>\$ 286,614</b>	<b>\$ 56,479</b>	<b>\$ 19,237</b>

	December 31, 2020						
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities <sup>(1)</sup>	Long-term Marketable Securities <sup>(2)</sup>
<b>Level 1:</b>							
Cash and cash equivalents	\$ 268,269	\$ —	\$ —	\$ 268,269	\$ 268,269	\$ —	\$ —
Subtotal	268,269	—	—	268,269	268,269	—	—
<b>Level 2:</b>							
Certificates of deposit	2,986	7	—	2,993	—	2,993	—
Subtotal	2,986	7	—	2,993	—	2,993	—
<b>Total</b>	<b>\$ 271,255</b>	<b>\$ 7</b>	<b>\$ —</b>	<b>\$ 271,262</b>	<b>\$ 268,269</b>	<b>\$ 2,993</b>	<b>\$ —</b>

(1) The Company's short-term marketable securities mature in one year or less.

(2) The Company's long-term marketable securities mature between one year and two years.

See Note 2 to these unaudited condensed consolidated financial statements for additional discussion regarding the Company's fair value measurements.

## 5. Balance Sheet Components

### *Property and Equipment, Net*

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

	September 30, 2021	December 31, 2020
	(Unaudited)	
Construction in progress	\$ 70,211	\$ 23,031
Leasehold improvements	5,723	4,631
Furniture and fixtures	891	870
Computer equipment and software	85	82
Laboratory and manufacturing equipment	5,485	4,630
Total property and equipment	82,395	33,244
Accumulated depreciation and amortization	(3,753)	(2,368)
Property and equipment, net	\$ 78,642	\$ 30,876

Depreciation expense was \$474 thousand and \$1.4 million for the three and nine months ended September 30, 2021 and \$399 thousand and \$1.1 million three and nine months ended September 30, 2020, respectively.

### *Accrued Expenses and Other Current Liabilities*

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

	September 30, 2021	December 31, 2020
	(Unaudited)	
Accrued preclinical and clinical expenses	\$ 3,743	\$ 1,735
Accrued professional fees	1,903	642
Accrued payroll and benefits	1,799	1,486
Accrued taxes	32	40
Accrued construction in progress	22,064	1,049
Accrued financing costs	447	131
Other current liabilities	11	26
Total	\$ 29,999	\$ 5,109

## 6. Commitments and Contingencies

### *Significant Contracts and Agreements*

#### *Lease Agreements*

On May 26, 2016, the Company signed an operating lease for laboratory and office space that commenced in June 2016 and expired on October 31, 2017 (the "2016 Lease"). The 2016 Lease has been amended several times to increase the area leased, which currently consists of approximately 44,000 square feet. As a result of the lease amendments, the lease expiration date was extended to October 31, 2031.

On December 26, 2019, we entered into a lease agreement for our second commercial gene therapy facility ("ASTRA") in the Pittsburgh, Pennsylvania area ("ASTRA lease") with Northfield I, LLC (the "Landlord" or "Northfield") with an initial lease term that expired on October 31, 2035. The ASTRA lease contained an option ("Purchase Option") to purchase the building, related improvements and take corresponding assignment of the Landlord's rights under its existing Ground Lease (the

"Ground Lease"). A cash contribution in the amount of \$2.4 million was paid to escrow on January 21, 2020. The contribution was intended to reduce the amount of the building construction costs and had the effect of reducing the base rental rate of the lease and as such, was recorded as prepaid rent in the consolidated balance sheet at the time of payment.

On October 5, 2020, the Company was provided with notice that the initial delivery conditions of the building had been met, including completion of the building shell, interior slab, and exterior doors, and on October 15, 2020, the Company gave the Landlord notice of its intent to purchase ASTRA for approximately \$9.4 million, subject to the parties entering into a commercially reasonable purchase and sale agreement. As a result of the Company's ability to exercise its option to purchase ASTRA, the Company obtained control over the construction in progress of ASTRA as of October 5, 2020. The Company recorded a \$10.0 million CIP asset and a corresponding build to suit lease liability related to the costs incurred by the Landlord, offset by the previous cash contribution of \$2.4 million.

On January 29, 2021, the Company entered into a Purchase and Sale Agreement ("PSA") for ASTRA with Northfield related to the purchase option exercised by the Company on October 15, 2020, for a purchase price of \$9.4 million. The Company held approximately \$1.5 million on deposit with Northfield under the existing lease agreement and applied this deposit as a credit against the purchase price at closing. On February 1, 2021, Northfield delivered the space as substantially complete and made the space available for access by the Company, thus triggering lease commencement. As a result, the Company concluded that this transaction did not qualify for sale-leaseback accounting because it did not meet the definition of a sale. As control did not transfer to the Lessor at lease commencement, the transaction continued to be accounted for as construction in progress and a financing obligation. On March 5, 2021, the purchase closed and the Company determined that reclassification of the construction in progress to buildings and leasehold improvements was not appropriate as the interior of the building was not yet ready for its intended use. The building continues to be held under construction in progress as of September 30, 2021. The interior of the building is currently under construction and is expected to be completed and validated in 2022. From construction completion to the closing of the purchase, the Company recognized interest expense to accrete the financial obligation to a balance that equaled the cash consideration that was paid upon the close of purchase. For more information about the expected construction costs associated with ASTRA, see "ASTRA Contractual Obligations" below.

As part of the transaction, the Company also became the accounting owner of the Ground Lease, due to obtaining control over ASTRA, and recorded the applicable operating right-of-use asset and corresponding lease liability as of October 5, 2020. When the PSA was finalized, the Company took assignment of the Lessor's Ground Lease, in accordance with the Purchase Option, of which lease payments are based on annual payments of \$82 thousand, and are subject to a cumulative 10% escalation clause every 5 years through 2071.

As of September 30, 2021, future minimum commitments under the Company's operating leases were as follows (in thousands):

	<b>Operating Leases</b>
2021 (remaining three months)	\$ 255
2022	1,094
2023	1,114
2024	1,135
2025	1,162
Thereafter	12,786
Future minimum operating lease payments	\$ 17,546
Less: Interest	9,444
Present value of lease liability	\$ 8,102

Supplemental condensed consolidated balance sheet information related to leases is as follows:

	(unaudited)			
	September 30, 2021		December 31, 2020	
Operating leases:				
Right-of-use assets	\$	7,342	\$	3,298
Current portion of lease liability		1,023		638
Lease liability		7,079		3,308
Total lease liability	\$	8,102	\$	3,946
Weighted average remaining lease term, in years		14.6		16.4
Weighted average discount rate		9.2 %		9.4 %

The Company recorded operating lease costs of \$339 thousand and \$836 thousand for the three and nine months ended September 30, 2021 and \$148 thousand and \$458 thousand for the three and nine months ended September 30, 2020, respectively, and variable lease costs of \$65 thousand and \$183 thousand for the three and nine months ended September 30, 2021 and \$60 thousand and \$88 thousand for the three and nine months ended September 30, 2020, respectively.

#### *Agreements with Contract Manufacturing Organizations and Contract Research Organizations*

The Company has entered into various agreements with Contract Manufacturing Organizations ("CMOs") for the manufacture of sterile excipients that are formulated with in-house produced vectors as part of the final drug product applied in certain of our clinical trials. The Company has also entered into agreements with third-party Contract Research Organizations ("CROs") to provide research and development services to further the Company's analysis of its product candidates. The agreements entered into with the CMOs and CROs provide the terms and conditions for their respective services, which may include research and development activities, storage, packaging, labelling, and/or testing of our preclinical and clinical-stage products. The Company is obligated to make milestone payments under certain of these agreements. The estimated remaining commitment as of September 30, 2021 under these agreements is approximately \$2.6 million. The Company may also be responsible for the payment of a monthly service fee for project management services for the duration of any agreements. The Company has incurred expenses under these agreements of \$744 thousand and \$3.3 million for the three and nine months ended September 30, 2021 and \$1.3 million and \$2.3 million for the three and nine months ended September 30, 2020, respectively.

#### *Commercial Preparedness Activities*

The Company has contracted with various third parties to facilitate, coordinate and perform agreed upon commercial preparedness and market research activities relating to our lead product candidate, B-VEC. These contracts typically call for the payment of fees for services upon the achievement of certain milestones. The estimated remaining commitment as of September 30, 2021 is \$3.9 million. The Company has incurred expenses under these activities of \$1.8 million and \$4.1 million for the three and nine months ended September 30, 2021 and \$582 thousand and \$1.1 million for the three and nine months ended September 30, 2020, respectively.

#### *ASTRA Contractual Obligations*

The Company has contracted with various third parties to construct our second cGMP facility, ASTRA. Additionally, we have entered into various non-cancellable purchase agreements for long-lead materials to help avoid potential schedule disruptions or material shortages. These contracts typically call for the payment of fees for services or materials upon the achievement of certain milestones. The estimated remaining commitment as of September 30, 2021 is \$39.4 million. The Company has included costs incurred to-date associated with ASTRA within construction in progress as of September 30, 2021.

On June 30, 2021, the Company entered into a Standard Form of Contract for Construction and the corresponding General Conditions of the Contract for Construction (collectively, the "Agreement") with The Whiting-Turner Contracting Company ("Whiting-Turner"), pursuant to which Whiting-Turner is constructing and managing the construction of ASTRA. Subject to certain conditions in the Agreement, the Company will pay Whiting-Turner a contract price consisting of the cost of work plus a fee equal to 1.75% of the cost of work, subject to a guaranteed maximum price to be agreed upon in an amendment to the Agreement at a later date.

Effective September 13, 2021, the Company entered into a guaranteed maximum price amendment (the "Amendment") to the Agreement to set forth the guaranteed maximum price, as well as the date by which Whiting-Turner is to achieve

Substantial Completion (as defined in the Agreement). Under the Amendment, the guaranteed maximum price to be paid by the Company is \$78.9 million, subject to certain additions and deductions by change orders as provided by the Agreement, and the date of Substantial Completion of Whiting-Turner's work under the Agreement is March 30, 2022. Whiting-Turner's work under the Agreement represents a portion of the work necessary to complete construction of the ASTRA facility and, therefore the date of Substantial Completion of Whiting-Turner's work under the Agreement does not equate to the date of completion of ASTRA. The guaranteed maximum price under the Agreement constitutes only a portion of the total estimated cost of building and equipping ASTRA.

### *Legal Proceedings*

On May 1, 2020, a complaint was filed against us in the United States District Court for the Western District of Pennsylvania by PeriphaGen, Inc. ("PeriphaGen"), which also named our Chief Executive Officer and Chief Operating Officer, Krish Krishnan and Suma Krishnan, respectively. The complaint alleges breach of contract and misappropriation of trade secrets, which secrets the plaintiff asserts were used to develop our product candidates, including the vector backbones, and our STAR-D platform. We answered the complaint on June 26, 2020 by denying the allegations and brought a counterclaim asking the court to declare that we did not misappropriate PeriphaGen's trade secrets or confidential information, and to further declare that we are the rightful and sole owner of our product candidates and STAR-D platform. In addition, we filed a third-party complaint against two principals of PeriphaGen, James Wechuck and David Krisky, alleging breach of contract and seeking contribution and indemnification from them in the event PeriphaGen is awarded damages. On July 29, 2020, PeriphaGen filed its response to our answer and counterclaim, denying the allegations in the counterclaim. On the same day, Messrs Wechuck and Krisky filed a motion to dismiss the third-party complaint on various grounds, and we opposed the motion. On December 1, 2020, the court ruled on Messrs. Wechuck and Krisky's motion to dismiss our third-party complaint. The court determined that our claims for contribution and indemnification based on PeriphaGen's state law claims for unfair competition and misappropriation of trade secrets can proceed. Our breach of contract claim will also go forward in full. Fact discovery is ongoing.

PeriphaGen is seeking monetary damages, injunctive relief, attorneys' fees and costs. While we are unable to provide any assurances as to the ultimate outcome of the case, we believe the allegations in the complaint are without merit, and we intend to vigorously defend against them. We are currently unable to estimate the costs and timing of any litigation, including any potential damages if PeriphaGen were to prevail on its claims.

The Company has received insurance proceeds during fiscal year 2021 relating to legal defense costs and expenses associated with the PeriphaGen litigation. During the three and nine months ended September 30, 2021, the Company has received \$954 thousand of insurance proceeds and an additional \$619 thousand has been approved for reimbursement, which is expected to be received in November 2021 and has been recorded as a receivable within Prepaid Expenses and Other Current Assets on the Condensed Consolidated Balance Sheet as management determined that the amount was probable of collection. The reimbursements have been recorded as an offset to our legal fees included in General and Administrative expenses on the Condensed Consolidated Statements of Operations and within Operating Activities on the Condensed Consolidated Statements of Cash Flows.

## **7. Capitalization**

### *Sale of Common Stock*

On February 1, 2021, the Company completed a public offering of 2,211,538 shares of its common stock, including 288,461 shares purchased by the underwriters, at \$65.00 per share. Net proceeds to the Company from the offering were \$134.9 million after deducting underwriting discounts and commissions of approximately \$8.6 million, and other offering expenses payable by the Company of \$198 thousand.

On December 31, 2020, the Company entered into a sales agreement (the "Sales Agreement") with Cowen and Company, LLC ("Cowen") with respect to an at-the-market equity offering program ("ATM Program"), under which Cowen will act as the Company's agent and/or principal and may issue and sell from time to time, during the term of the Sales Agreement, shares of our common stock, par value \$0.0001 per share, having an aggregate offering price up to \$150.0 million ("Placement Shares"). Related offering expenses payable by the Company were \$172 thousand. The issuance and sale of the Placement Shares by the Company under the Sales Agreement will be made pursuant to the Company's effective "shelf" registration statement on Form S-3. During the nine months ended September 30, 2021, 262,500 shares of common stock were issued pursuant to the ATM Program at a weighted average price of \$66.50 per share for net proceeds of \$16.9 million after deducting underwriting discounts and commissions of approximately \$524 thousand, resulting in a remaining \$132.5 million available for issuance under the ATM Program.



On May 21, 2020, the Company completed a public offering of 2,275,000 shares of its common stock to the public at \$55.00 per share. Net proceeds to the Company from the offering were \$117.2 million after deducting underwriting discounts and commissions of approximately \$7.5 million, and other offering expenses payable by the Company of approximately \$463 thousand.

## 8. Stock-Based Compensation

### Stock Options

Stock options granted to employees and non-employees vest ratably over four-year periods and stock options granted to directors of the company vest ratably over one year to four-year periods. Stock options have a life of ten years.

The Company granted 322,500 and 1,122,450 stock options to employees and directors of the Company during the three and nine months ended September 30, 2021, respectively, and 195,850 and 740,250 stock options to employees and directors of the Company during the three and nine months ended September 30, 2020, respectively.

The Company granted 50,000 and zero stock options to non-employees during the three and nine months ended September 30, 2021 and 2020, respectively.

The following table summarizes the Company's stock option activity:

	Stock Options Outstanding	Weighted- average Exercise Price	Weighted- average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (In thousands) <sup>(1)</sup>
Outstanding at December 31, 2020	853,614	\$ 40.31	9.0	\$ 16,804
Granted	1,172,450	\$ 67.15		
Exercised	(49,726)	\$ 38.11		
Cancelled or forfeited	(172,000)	\$ 61.28		
Expired	(2,500)	\$ 78.89		
Outstanding at September 30, 2021	<u>1,801,838</u>	<u>\$ 55.78</u>	<u>9.1</u>	<u>\$ 9,456</u>
Exercisable at September 30, 2021	<u>265,843</u>	<u>\$ 30.02</u>	<u>7.4</u>	<u>\$ 6,087</u>

(1) Aggregate intrinsic value represents the difference between the closing stock price of our common stock on September 30, 2021 and the exercise price of outstanding in-the-money options.

The total intrinsic value (the amount by which the fair market value exceeds the exercise price) of stock options exercised during the three and nine months ended September 30, 2021 was \$348 thousand and \$1.2 million, respectively, and during the three and nine months ended September 30, 2020 was \$1.7 million and \$2.9 million, respectively.

The weighted-average grant-date fair value per share of options granted to employees and directors during the three and nine months ended September 30, 2021 was \$35.00 and \$43.73, respectively, and during the three and nine months ended September 30, 2020 was \$27.44 and \$30.58, respectively.

The weighted-average grant-date fair value per share of options granted to non-employees during the three and nine months ended September 30, 2021 was \$33.93 and \$33.93, respectively, and during the three and nine months ended September 30, 2020 was zero, respectively.

There was \$51.9 million of unrecognized stock-based compensation expense related to employees and directors' option awards that is expected to be recognized over a weighted-average period of 3.3 years as of September 30, 2021.

There was \$1.7 million of unrecognized stock-based compensation expense related to non-employees' option awards that is expected to be recognized over a weighted-average period of 3.9 years as of September 30, 2021.

The Company has recorded aggregate stock-based compensation expense related to the issuance of stock option awards in the condensed consolidated statements of operations for the three and nine months ended September 30, 2021 and 2020 as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
	(unaudited)		(unaudited)	
Research and development	\$ 673	\$ 344	\$ 2,273	\$ 714
General and administrative	2,502	1,012	6,742	1,988
Total stock-based compensation	\$ 3,175	\$ 1,356	\$ 9,015	\$ 2,702

We capitalize the portion of stock-based compensation that relates to work performed on the construction of new buildings. There was \$79 thousand and \$182 thousand of stock-based compensation that was capitalized in the three and nine months ended September 30, 2021, respectively, and zero of stock-based compensation that was capitalized in the three and nine months ended September 30, 2020, respectively.

The Company recorded stock-based compensation expense of \$3.2 million and \$9.0 million for the three and nine months ended September 30, 2021, respectively, and \$1.4 million and \$2.7 million for the three and nine months ended September 30, 2020, respectively. The fair value of options was estimated at the date of grant using the Black-Scholes valuation model with the following weighted-average assumptions 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
Expected stock price volatility	73 %	75 %	73 %	75 %
Expected term of the award (years)	6.23	6.20	6.19	6.20
Risk-free interest rate	1.13 %	0.34 %	1.05 %	0.66 %
Weighted average exercise price	\$ 53.69	\$ 41.89	\$ 67.15	\$ 46.46
Forfeiture rate	— %	10.85 %	— %	10.85 %

#### Restricted Stock Awards

Restricted stock awards ("RSAs") granted to employees vest ratably over a four-year period. Restricted stock awards have a life of ten years.

The Company granted zero and 98,800 RSAs to employees of the Company during the three and nine months ended September 30, 2021, respectively, and zero RSAs to employees of the Company during the three and nine months ended September 30, 2020.

The following table summarizes the Company's RSA activity:

	Number of Shares	Weighted Average Grant Date Fair Value
Non-vested RSAs as of December 31, 2020	—	\$ —
Granted	98,800	\$ 78.89
Vested	—	\$ —
Forfeited	—	\$ —
Non-vested RSAs as of September 30, 2021	98,800	\$ 78.89

As of September 30, 2021, 98,800 RSAs were outstanding. The fair value of each restricted stock award was \$78.89 reflecting the closing price of our common stock on the grant date. The Company recorded stock-based compensation expense related to RSAs of \$491 thousand and \$1.2 million for the three and nine months ended September 30, 2021, respectively, and zero for the three and nine months ended September 30, 2020, within general and administrative expenses in the accompanying condensed consolidated statements of operations (in thousands):

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2021</b>	<b>2020</b>	<b>2021</b>	<b>2020</b>
	<b>(unaudited)</b>		<b>(unaudited)</b>	
General and administrative	\$ 491	\$ —	\$ 1,159	\$ —
Total stock-based compensation	\$ 491	\$ —	\$ 1,159	\$ —

Shares remaining available for grant under the Company's stock incentive plan were 1,381,481, with a sublimit for incentive stock options of 379,921, at September 30, 2021.

#### 9. Related Party Transactions

In December 2019, the Company advanced \$420 thousand to a member of our management team to cover the personal payroll and income taxes on their taxable income from NSO exercises. This employee repaid the Company in full in January 2020.

#### 10. Subsequent Events

The Company evaluates events or transactions that occur after the balance sheet date, but prior to the issuance of the financial statements, to identify matters that require disclosure. The Company concluded that no subsequent events have occurred that would require recognition or disclosure in the condensed consolidated financial statements.

## 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 together with the unaudited condensed consolidated financial statements and related notes included in Item 1 of Part I of this Quarterly Report on Form 10-Q and with the audited financial statements and the related notes included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020, as filed with the SEC, on March 1, 2021.*

### SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "seek," "should," "target," "will," "would," or similar expressions and the negatives of those terms. These statements relate to future events or to our future operating or financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. Some of such factors include, but are not limited to:

- changes in expectations with respect to the initiation, timing, progress and results of preclinical and clinical trials for B-VEC, KB105, KB104, KB407, KB408, KB301, KB303 and any other product candidates, including the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs and expenses;
- the continuing impact that the COVID-19 pandemic and measures implemented to prevent its spread may have on our business operations, access to capital, research and development activities, and preclinical and clinical trials for our product candidates;
- the timing, scope or results of regulatory filings and approvals, including timing of final US Food and Drug Administration ("FDA"), marketing and other regulatory approval of our product candidates;
- our ability to achieve certain accelerated or orphan drug designations from the FDA;
- changes in our estimates regarding the potential market opportunity for B-VEC, KB105, KB104, KB407, KB408, KB301, KB303 and any other product candidates;
- our ability to raise capital to fund our operations;
- increased costs associated with our research and development programs for our product candidates;
- our general and administrative expenses;
- risks related to our ability to successfully develop and commercialize our product candidates, including B-VEC, KB105, KB104, KB407, KB408, KB301, KB303 and our other product candidates;
- our ability to identify and develop new product candidates;
- our ability to identify, recruit and retain key personnel;
- risks related to our commercialization, marketing and manufacturing capabilities and strategy;
- our ability of our business model, strategic plans for our business, product candidates and technology;
- the scalability and commercial viability of our proprietary manufacturing methods and processes;
- the rate and degree of market acceptance and clinical utility of our product candidates and gene therapy, in general;
- our competitive position;
- our intellectual property position and our ability to protect and enforce our intellectual property;
- our financial performance;
- developments and projections relating to our competitors and our industry;
- our ability to establish and maintain collaborations or obtain additional funding;
- our estimates regarding expenses, future revenue, capital requirements and needs for or ability to obtain additional financing;

- risks related to our ongoing litigation;
- our ability to successfully resolve any intellectual property or other claims that have been brought against us to date and may be brought against us in the future;
- global economic conditions; and
- the impact of changes in laws and regulations.

Forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in “Risk Factors” elsewhere in this Form 10-Q and in other filings we make with the SEC from time to time. Moreover, we operate in a very competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Quarterly Report on Form 10-Q may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent our management’s beliefs and assumptions only as of the date of this Quarterly Report. You should read this Quarterly Report completely and with the understanding that our actual future results may be materially different from what we expect.

Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

Throughout this Form 10-Q, unless the context requires otherwise, all references to “Krystal,” “the Company,” “we,” “our,” “us” or similar terms refer to Krystal Biotech, Inc., together with its consolidated subsidiaries.

### **Overview**

We are a clinical stage biotechnology company leading the field of redosable gene delivery for the treatment of serious rare diseases. Using our patented platform that is based on engineered HSV-1, we create vectors that efficiently deliver therapeutic transgenes to cells of interest in multiple organ systems. The cell’s own machinery then transcribes and translates the encoded effector to treat or prevent disease. We formulate our vectors for non-invasive or minimally invasive routes of administration at a doctor’s office or potentially in the patient’s home by a healthcare professional. Our goal is to develop easy-to-use medicines to dramatically improve the lives of patients living with rare diseases. Our innovative technology platform is supported by in-house, commercial scale cGMP manufacturing capabilities.

## Our Product Candidates

The following table summarizes information regarding our product candidates in various stages of clinical and preclinical development:

	Product	Protein	Indication	Discovery	Preclinical	Phase 1/2	Phase 3	Key Upcoming Milestone	Ownership
Dermatology	<b>B-VEC</b> <sup>†§</sup>	Type VII collagen	Dystrophic EB	→				Top line Phase 3 data in 4Q21	Krystal
	<b>KB105</b> <sup>†§</sup>	Transglutaminase 1 (TGM1)	TGM1-deficient ARCI	→				Resume dosing in Phase 2 study in 2022	Krystal
	<b>KB104</b> <sup>‡</sup>	Serine Peptidase Inhibitor Kazal Type 5 (SPINK5)	Netherton Syndrome	→				File IND in 2022	Krystal
	<b>KB1XX</b>	Undisclosed programs		→					Krystal
	<b>KB301</b>	Type III collagen	Aesthetic skin conditions	→				Phase 1 efficacy data in 1Q22	JEUNE
	<b>KB302</b>	Type I collagen	Aesthetic skin conditions	→					JEUNE
	<b>KB303</b>	Elastin	Aesthetic skin conditions	→					JEUNE
	<b>KB304</b>	Type III collagen & Elastin	Aesthetic skin conditions	→					JEUNE
	<b>KB5XX</b>	Vector encoded antibodies	Chronic skin conditions	→					Krystal
	Respiratory	<b>KB407</b> <sup>†‡</sup>	Cystic fibrosis transmembrane conductance regulator (CFTR)	Cystic fibrosis	→				Initiate Phase 1 study in 4Q21
<b>KB408</b>		Alpha-1 antitrypsin (AAT)	alpha-1 antitrypsin deficiency	→					Krystal
<b>KB4XX</b>		Undisclosed programs		→				Krystal	

All pipeline compounds are investigational, being evaluated in clinical or pre-clinical studies.

†: FDA Orphan Drug Designation;  
‡: FDA Rare Pediatric Disease Designation;  
•: Fast-track Designation;

Δ: FDA RMAT designation;  
‡: EMA Orphan Drug Designation;  
§: EMA PRIME Designation.

Rare disease

More prevalent conditions

There can be no assurance that the upcoming milestones will be met on the expected timeline or at all.

## Pipeline Highlights and Recent Developments

- B-VEC is a topical gel containing our novel vector designed to deliver two copies of the *COL7A1* transgene for the treatment of dystrophic epidermolysis bullosa ("DEB"), a serious rare skin disease caused by missing or mutated type VII collagen protein ("COL7"). The randomized, double-blind, placebo-controlled GEM-3 pivotal study was designed to evaluate topical B-VEC as compared to placebo in DEB patients. On October 25, 2021,

we announced completion of the GEM-3 study, and we expect to announce top line data in 4Q21. Details of the pivotal study can be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) under NCT identifier NCT04491604. During 2Q21, we began enrolling patients into an open label extension ("OLE") study, including patients who participated in the Phase 3 study, as well as new participants who meet all enrollment criteria. Details of the OLE study can be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) under NCT identifier NCT04917874. Nothing included on this website shall be deemed incorporated by reference into this Quarterly Report on Form 10-Q. On September 9, 2021, we announced that the U.S. FDA approved a compassionate use request from a physician for the use of topical (eye drop) B-VEC in the system of a single DEB patient after undergoing surgical removal of the scarred layer of the cornea.

- KB105 is a topical gel containing our novel vector designed to deliver two copies of the *TGM1* transgene for the treatment of TGM1-deficient autosomal recessive congenital ichthyosis ("TGM1-ARCI"), a serious rare skin disorder caused by missing or mutated TGM1 protein. A randomized, placebo-controlled Phase 1/2 study is ongoing. On July 1, 2021, we announced data from the fourth patient dosed in the trial, showing repeat topical KB105 dosing continued to be well tolerated with no adverse events or evidence of immune response. Details of the Phase 1/2 study can be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) under NCT identifier NCT04047732. Nothing included on this website shall be deemed incorporated by reference into this Quarterly Report on Form 10-Q.
- KB407 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the full-length *CFTR* transgene for the treatment of cystic fibrosis, a serious rare lung disease caused by missing or mutated cystic fibrosis transmembrane conductance regulator ("CFTR") protein. On September 29, 2021, we announced that the Bellberry Human Research Ethics Committee in Australia granted approval to conduct a Phase 1 clinical study of inhaled KB407 in patients with cystic fibrosis, and trial initiation is anticipated in 4Q21. More detailed data from the Good Laboratory Practice "GLP" toxicology and biodistribution study was presented at the virtual 2021 North American Cystic Fibrosis Conference that took place November 2-5, 2021.
- KB408 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the *SERPINA1* transgene, that encodes for normal human alpha-1 antitrypsin protein, for the treatment of alpha-1 antitrypsin deficiency. We presented preclinical pharmacology data for KB408 at the European Society of Gene & Cell Therapy Virtual Congress that was held October 19-22, 2021.
- KB104 is a topical gel formulation of our novel vector designed to deliver two copies of the *SPINK5* transgene for the treatment of Netherton Syndrome, a debilitating autosomal recessive skin disorder caused by missing or mutated SPINK5 protein. We expect to initiate a Phase 1 clinical study in 2022.

We are also leveraging the ability of our platform to deliver proteins of interest to cells in the skin in the context of aesthetic medicine via our wholly owned subsidiary Jeune Aesthetics, Inc. ("Jeune"). A summary description of Jeune's key product candidate and its status is as follows:

- KB301 is a solution formulation of our novel vector for intradermal injection designed to deliver two copies of the *COL3A1* transgene to address signs of aging or damaged skin caused by declining levels of, or damaged proteins within the extracellular matrix, including type III collagen. A Phase 1 study is currently ongoing. On August 2, 2021, Jeune announced the dosing of the first patient in the second Cohort of the PEARL-1 study. This cohort is a randomized, double-blind, saline controlled evaluation of safety and efficacy of KB301 for the improvement of skin quality. Enrollment of this cohort completed in October 2021, and Jeune expects to announce initial data from Cohort 2 in early 2022. Details of the Phase 1 study can be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) under NCT identifier NCT04540900. Nothing included on this website shall be deemed incorporated by reference into this Quarterly Report on Form 10-Q

Jeune has several other aesthetic medicine product candidates in various stages of preclinical development.

#### **Business Highlights and Recent Developments**

- On September 15, 2021, we announced the appointment of Laurent Goux as the General Manager of Europe.
- On October 12, 2021, we announced a collaboration with GeneDx, Inc., a wholly-owned subsidiary of BioReference Laboratories, Inc., an OPKO Health company, to offer no-charge genetic testing for all types of Epidermolysis Bullosa (EB). The goal of the program, called Krystal Decode DEB™, is to help patients with the dystrophic form of this genetic condition, also known as DEB, get a definitive diagnosis sooner, with highly accurate results obtained with a blood or cheek swab sample.

#### **COVID-19 Update**

The COVID-19 pandemic has prompted governments and businesses to take unprecedented measures, such as restrictions on travel and business operations, temporary closures of businesses, and quarantines. In an effort to slow the spread of the virus, The Commonwealth of Pennsylvania where the Company's primary offices, laboratory and manufacturing spaces are located, enacted stay-at-home orders, and sweeping restrictions to travel were initiated by corporations and governments. Although these restrictions have been lifted, it is not known at this time whether they will be reestablished or the extent to which the Company will be impacted. The degree of the pandemic's effect on the Company's clinical, operational and financial performance will depend on future developments, including additional protective measures that may be implemented by governmental authorities or the Company to protect its employees, or by investigators, caregivers or patients to minimize exposure, all of which are uncertain and difficult to predict. While to date the impact of the pandemic on our business and clinical trials has been minimal and the increased vaccination rates in the U.S. are encouraging, we will continue to assess the potential impact of the coronavirus disease ("COVID-19") pandemic on our business and operations, including our supply chain and preclinical and clinical trial activities. For additional information regarding the impact of the coronavirus pandemic, please see "Risk Factor - Business interruptions resulting from the COVID-19 outbreak or similar public health crises could cause a disruption of the development efforts of our product candidates and adversely impact our business."

## **Financial Overview**

### ***Revenue***

We currently have no approved products for commercial marketing or sale and have not generated any revenue from the sale of products or other sources to date. In the future, we may generate revenue from product sales, royalties on product sales, or license fees, milestones, or other upfront payments if we enter into any collaborations or license agreements. We expect that our future revenue will fluctuate from quarter to quarter for many reasons, including the uncertain timing and amount of any such payments and sales.

### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred to advance our preclinical and clinical candidates, which include:

- expenses incurred under agreements with contract manufacturing organizations, consultants and other vendors that conduct our preclinical activities;
- costs of acquiring, developing and manufacturing clinical trial materials and lab supplies;
- facility costs, depreciation and other expenses, which include direct expenses for rent and maintenance of facilities and other supplies; and
- payroll related expenses, including stock-based compensation expense.

We expense internal research and development costs to operations as incurred. We expense third party costs for research and development activities, such as the manufacturing of preclinical and clinical materials, based on an evaluation of the progress to completion of specific tasks such as manufacturing of drug substance, fill/finish and stability testing, which is provided to us by our vendors.

We expect our research and development expenses will increase as we continue the manufacturing of preclinical and clinical materials and manage the clinical trials of, and seek regulatory approval for, our product candidates and expand our product portfolio. In the near term, we expect that our research and development expenses will increase as we continue with our pivotal Phase 3 clinical trial for B-VEC, conduct our ongoing Phase 1/2 clinical trial for KB105, conduct our phase 1 safety study for KB301 and incur preclinical expenses for our other product candidates. Due to the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration, costs and timing of our clinical trials, and, as a result, the actual costs to complete our clinical trials may exceed the expected costs.

### ***General and Administrative Expenses***

General and administrative expenses consist principally of professional fees associated with corporate and intellectual property-related legal expenses, consulting and accounting services, facility-related costs and expenses associated with obtaining and maintaining patents. Other general and administrative costs include stock-based compensation and travel expenses.

We anticipate that our general and administrative expenses will increase in the future to support the continued research and development of our product candidates and to operate as a public company. These increases will likely include increased



costs for insurance, costs related to the hiring of additional personnel and payments to outside consultants, lawyers and accountants, among other expenses. Additionally, if and when we believe a regulatory approval of our first product candidate appears likely, we anticipate that we will increase our salary and personnel costs and other expenses as a result of our preparation for commercial operations.

#### ***ASTRA Capital Expenditures***

On March 5, 2021, we closed on the purchase of the building that was constructed to house our second cGMP facility, ASTRA. We are currently in the process of constructing the interior build-out of this facility and we have entered into a contract with Whiting-Turner who will manage the construction of ASTRA. Further, we have entered into various non-cancellable purchase agreements for long-lead materials to help avoid potential schedule disruptions or material shortages. These contracts typically call for the payment of fees for services or materials upon the achievement of certain milestones. We expect to continue to incur significant capital expenditures related to ASTRA as we construct and validate this facility, which is expected to be completed in 2022.

#### ***Interest Income***

Interest income consists primarily of income earned from our cash, cash equivalents and investments.

#### ***Interest Expense***

Interest expense consists primarily of non-cash interest expense recognized to accrete the build to suit financial obligation to a balance that equaled the cash consideration that was paid upon the close of the purchase of ASTRA.

#### ***Critical Accounting Policies, Significant Judgments and Estimates***

There have been no significant changes during the three and nine months ended September 30, 2021 to our critical accounting policies, significant judgments and estimates as disclosed in our management's discussion and analysis of financial condition and results of operations included in our Annual Report on Form 10-K for the year ended December 31, 2020.

## Results of Operations

### Three Months Ended September 30, 2021 and 2020

(In thousands)	Three Months Ended September 30,		Change
	2021	2020	
	(unaudited)		
<b>Expenses</b>			
Research and development	\$ 6,080	\$ 5,100	\$ 980
General and administrative	9,572	4,580	4,992
Total operating expenses	15,652	9,680	5,972
Loss from operations	(15,652)	(9,680)	(5,972)
<b>Other Income</b>			
Interest and other income, net	63	70	(7)
Net loss	\$ (15,589)	\$ (9,610)	\$ (5,979)

#### Research and Development Expenses

Research and development expenses increased \$980 thousand in the three months ended September 30, 2021 compared to the three months ended September 30, 2020. Higher research and development expenses were due to an increase in preclinical, clinical and pre-commercial manufacturing activities of \$538 thousand, payroll related expenses of \$363 thousand, which were primarily driven by an increase in headcount to support overall growth, and includes a \$327 thousand increase in stock-based compensation, software related costs of \$167 thousand, and other research and development expenses of \$191 thousand, primarily due to depreciation and rent. These increases were offset by decrease in outsourced research and development activities of approximately \$279 thousand.

#### General and Administrative Expenses

General and administrative expenses increased \$5.0 million in the three months ended September 30, 2021 as compared to the three months ended September 30, 2020. Higher general and administrative spending was due largely to increases in payroll related expenses of approximately \$3.1 million, which was primarily driven by an increase in headcount to support overall growth, and includes a \$2.0 million increase in stock-based compensation, commercial preparedness expenses of approximately \$1.2 million, medical affairs costs of \$101 thousand, software related costs of \$453 thousand, and other administrative expenses of \$422 thousand, primarily due to rent and insurance costs. These increases were offset by a decrease in legal and professional fees of approximately \$316 thousand, which includes \$1.6 million of insurance proceeds.

#### Other Income

Interest and other income for the three months ended September 30, 2021 and 2020 was \$63 thousand and \$70 thousand, respectively, and consisted of interest and dividend income earned from our cash, cash equivalents and investments.

*Nine Months Ended September 30, 2021 and 2020*

	Nine Months Ended September 30,		Change
	2021	2020	
(In thousands)	(unaudited)		
<b>Expenses</b>			
Research and development	\$ 18,875	\$ 12,264	\$ 6,611
General and administrative	27,524	10,315	17,209
Total operating expenses	46,399	22,579	23,820
Loss from operations	(46,399)	(22,579)	(23,820)
<b>Other Income (Expense)</b>			
Interest and other income, net	127	795	(668)
Interest expense	(1,492)	—	(1,492)
Net loss	<u>\$ (47,764)</u>	<u>\$ (21,784)</u>	<u>\$ (25,980)</u>

*Research and Development Expenses*

Research and development expenses increased \$6.6 million in the nine months ended September 30, 2021 compared to the nine months ended September 30, 2020. Higher research and development expenses were due to an increase in outsourced research and development activities of approximately \$2.0 million, preclinical, clinical and pre-commercial manufacturing activities of \$1.8 million, payroll related expenses of \$1.9 million, which was primarily driven by an increase in headcount to support overall growth, and includes a \$1.6 million increase in stock-based compensation, travel related expenses associated with our clinical trial sites of \$199 thousand, software related costs of \$212 thousand, and other research and development expenses of \$611 thousand, primarily due to depreciation and rent.

*General and Administrative Expenses*

General and administrative expenses increased \$17.2 million in the nine months ended September 30, 2021 as compared to the nine months ended September 30, 2020. Higher general and administrative spending was due largely to increases in payroll related expenses of approximately \$9.0 million, which was primarily driven by an increase in headcount to support overall growth, and includes a \$5.9 million increase in stock-based compensation, commercial preparedness expenses of approximately \$2.2 million, medical affairs costs \$437 thousand, software related costs of \$715 thousand, legal and professional fees of approximately \$3.6 million which includes \$1.6 million of insurance proceeds, insurance costs of \$408 thousand, and other administrative expenses of \$768 thousand, primarily due to rent.

*Other Income (Expense)*

Interest and other income for the nine months ended September 30, 2021 and 2020 was \$127 thousand and \$795 thousand, respectively, and consisted of interest and dividend income earned from our cash, cash equivalents and investments.

Interest expense for the nine months ended September 30, 2021 and 2020 was \$1.5 million and zero, respectively, and related to accretion of the financial obligation for the build to suit lease liability during the nine months ended September 30, 2021 to a balance that equaled the purchase consideration for ASTRA.

## Liquidity and Capital Resources

### Overview

At September 30, 2021, our cash, cash equivalents and short-term investments balance was approximately \$343.1 million. Since operations began, we have incurred operating losses. Our net losses were \$15.6 million and \$9.6 million for the three months ended September 30, 2021 and 2020 and \$47.8 million and \$21.8 million for the nine months ended September 30, 2021 and 2020, respectively. At September 30, 2021, we had an accumulated deficit of \$119.0 million. With the net proceeds raised from its public and private securities offerings, including the public offering completed on February 1, 2021 and the ATM Program, the Company believes that its cash, cash equivalents and short-term investments as of September 30, 2021 will be sufficient to allow the Company to fund its operations for at least 12 months from the filing date of this Form 10-Q.

As the Company continues to incur losses, a transition to profitability is dependent upon the successful development, approval and commercialization of our product candidates and the achievement of a level of revenues adequate to support the Company's cost structure. Furthermore, we expect to incur increasing costs associated with operating as a public company, meeting financial controls, satisfying regulatory and quality standards, maintaining product and clinical trials, and furthering our efforts around our current and future product candidates. The Company may never achieve profitability, and unless and until it does, the Company will continue to need to raise additional capital or obtain financing from other sources.

Costs related to clinical trials can be unpredictable and therefore there can be no guarantee that we will have sufficient capital to fund our continued clinical studies of B-VEC, KB105, KB301 or our planned preclinical studies for our other product candidates, or our operations. Further, we do not expect to generate any product revenues until 2022, at the earliest, assuming we receive marketing approval for B-VEC on the schedule we currently contemplate. While we are in the process of building out our internal vector manufacturing capacity, some of our manufacturing activities will be contracted out to third parties. Additionally, we currently utilize third-party contract research organizations to carry out our clinical development activities. As we seek to obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses as we prepare for product sales, marketing, manufacturing, and distribution. Our funds may not be sufficient to enable us to conduct pivotal clinical trials for, seek marketing approval for or commercially launch B-VEC, KB105, KB301 or any other product candidate. Accordingly, to obtain marketing approval for and to commercialize these or any other product candidates, we may be required to obtain further funding through public or private equity offerings, debt financings, collaboration and licensing arrangements or other sources. Adequate additional financing may not be available to us on acceptable terms, if at all. Our failure to raise capital when needed could have a negative effect on our financial condition and our ability to pursue our business strategy.

### Operating Capital Requirements

Our primary uses of capital are, and we expect will continue to be for the near future, compensation and related expenses, manufacturing costs for preclinical and clinical materials, third party clinical trial research and development services, laboratory and related supplies, clinical costs, legal and other regulatory expenses and general overhead costs. In order to complete the process of obtaining regulatory approval for any of our product candidates and to build the sales, manufacturing, marketing and distribution infrastructure that we believe will be necessary to commercialize our product candidates, if approved, we will require substantial additional funding.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the timeline and cost of our pivotal Phase 3 clinical trials for B-VEC;
- the progress, timing, results and costs of our ongoing Phase 1/2 clinical trials for KB105;
- the progress, results and costs of our Phase 1 clinical trials for KB301;
- the progress, timing and costs of manufacturing of B-VEC for our pivotal Phase 3 clinical trials;
- the continued development and the filing on an IND application for future product candidates;
- the initiation, scope, progress, timing, costs and results of drug discovery, laboratory testing, manufacturing, preclinical studies and clinical trials for any other product candidates that we may pursue in the future, if any;
- the costs of maintaining our own commercial-scale cGMP manufacturing facilities;

- the outcome, timing and costs of seeking regulatory approvals;
- the costs associated with the manufacturing process development and evaluation of third-party manufacturers;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, in the event we receive marketing approval for our current and future product candidates;
- the extent to which the costs of our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors;
- the costs of commercialization activities for our current and future product candidates if we receive marketing approval for such product candidates we may develop, including the costs and timing of establishing product sales, medical affairs, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, if any, revenue received from commercial sale of our current and future product candidates;
- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, maintenance, defense and enforcement of any patents or other intellectual property rights, including milestone and royalty payments and patent prosecution fees that we are obligated to pay pursuant to our license agreements;
- our current license agreements remaining in effect and our achievement of milestones under those agreements;
- our ability to establish and maintain collaborations and licenses on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

We expect that we will need to obtain substantial additional funding in order to receive regulatory approval and to commercialize our product candidates. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely affect our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of our product candidates, seek collaborators at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available, and relinquish or license, potentially on unfavorable terms, our rights to our product candidates that we otherwise would seek to develop or commercialize ourselves.

#### **Sources and Uses of Cash**

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

	<b>Nine Months Ended September 30,</b>	
	<b>2021</b>	<b>2020</b>
	<b>(unaudited)</b>	
Net cash used in operating activities	\$ (27,038)	\$ (18,059)
Net cash used in investing activities	(100,230)	(4,964)
Net cash provided by financing activities	145,613	117,878
Net increase in cash	<u>\$ 18,345</u>	<u>\$ 94,855</u>

#### **Operating Activities**

Net cash used in operating activities for the nine months ended September 30, 2021 was \$27.0 million and consisted primarily of a net loss of \$47.8 million adjusted for non-cash items primarily of depreciation and amortization and stock-based

compensation expense of \$12.2 million and build to suit interest expense of \$1.5 million, as well as increases in net operating liabilities of approximately \$7.1 million.

Net cash used in operating activities for the nine months ended September 30, 2020 was \$18.1 million and consisted primarily of a net loss of \$21.8 million adjusted for non-cash items of depreciation and amortization and stock-based compensation expense of approximately \$4.1 million, and decreases in net operating liabilities of approximately \$371 thousand.

#### *Investing Activities*

Net cash used in investing activities for the nine months ended September 30, 2021 was \$100.2 million and consisted primarily of expenditures of \$27.5 million on the build-out of our ASTRA facility, leasehold improvement of new office space, and purchases of computer and laboratory equipment, \$83.8 million on the purchase of short-term and long-term investments, partially offset by proceeds of \$11.0 million received from the maturities of short-term investments.

Net cash used in investing activities for the nine months ended September 30, 2020 was \$5.0 million and consisted primarily of purchases of \$3.2 million of short-term available-for-sale investment securities, and expenditures of \$7.6 million on the build-out of our ASTRA facility, leasehold improvement of new office space, and purchases of computer and laboratory equipment, partially offset by proceeds of \$5.9 million received from the maturities of short-term investments.

#### *Financing Activities*

Net cash provided by financing activities for the nine months ended September 30, 2021 was \$145.6 million and consisted primarily of proceeds of \$153.6 million received from our public offering, ATM Program and exercises of stock options, partially offset by expenditures of \$8.0 million used for the purchase of the ASTRA building.

On February 1, 2021 the Company completed a public offering of 2,211,538 shares of its common stock at \$65.00 per share. Net proceeds to the Company from the offering were \$134.9 million after deducting underwriting discounts and commissions of approximately \$8.6 million and other offering expenses of approximately \$198 thousand.

During the nine months ended September 30, 2021, pursuant to the ATM Program the Company issued 262,500 shares of common stock at a weighted average price of \$66.50 per share for net proceeds of \$16.9 million after deducting underwriting discounts and commissions of approximately \$524 thousand. The Company also incurred \$172 thousand of other offering expenses related to the ATM Program.

For the nine months ended September 30, 2021, the Company received proceeds of \$1.9 million from the exercise of stock options.

Net cash provided by financing activities for the nine months ended September 30, 2020 was \$117.9 million and was primarily from proceeds from our public offering in May 2020 of 2,275,000 shares of our common stock to the public at \$55.00 per share. Net proceeds to the Company from the offering were \$117.2 million after deducting underwriting discounts and commissions of approximately \$7.5 million and other offering expenses of approximately \$463 thousand.

#### **Off-Balance Sheet Arrangements**

We do not have any off-balance sheet arrangements as defined in the rules and regulations of the SEC.

#### **Contractual Obligations**

There have been no material changes to our contractual obligations as previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2020 other than as described in Note 6 “Commitments and Contingencies” of our condensed consolidated financial statements on this Form 10-Q.

#### **JOBS Act Accounting Election**

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (“the JOBS Act”). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, are subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. Beginning with our fiscal year ending December 31, 2022, we will cease to be an emerging growth company.

### **ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

#### **Qualitative and Quantitative Disclosures About Market Risk**

We had cash, cash equivalents and short-term investments of \$343.1 million at September 30, 2021, which consist primarily of money market, bank deposits, commercial paper, corporate bonds, and government agency securities. The investments in these financial instruments are made in accordance with an investment policy which specifies the categories, allocations and ratings of securities we may consider for investment. The primary objective of our investment activities is to preserve principal while at the same time maximizing the income we receive without significantly increasing risk. Some of the financial instruments in which we invest could be subject to market risk. This means that a change in prevailing interest rates may cause the value of the instruments to fluctuate. For example, if we purchase a security that was issued with a fixed interest rate and the prevailing interest rate later rises, the value of that security will probably decline. To minimize this risk, we intend to maintain a portfolio which may include cash, cash equivalents and short and long-term investment securities available-for-sale in a variety of securities which may include money market funds, government and non-government debt securities and commercial paper, all with various maturity dates. Based on our current investment portfolio, we do not believe that our results of operations or our financial position would be materially affected by an immediate change of 10% in interest rates.

We do not hold or issue derivatives, derivative commodity instruments or other financial instruments for speculative trading purposes. Further, we do not believe our cash, cash equivalents and short-term investments has significant risk of default or illiquidity. While we believe our cash, cash equivalents and short-term investments do not contain excessive risk, we cannot provide absolute assurance that any investments we make in the future will not be subject to adverse changes in market value. Our cash, cash equivalents and short and long-term investments are recorded at fair value.

### **ITEM 4. CONTROLS AND PROCEDURES**

#### **Evaluation of Disclosure Controls and Procedures**

Our Chief Executive Officer and our Chief Accounting Officer, with the participation of other members of the Company's management, have evaluated the effectiveness of the Company's "disclosure controls and procedures" (as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended ("Exchange Act")) as of the end of the period covered by this quarterly report, and our Chief Executive Officer and our Chief Accounting Officer have concluded that our disclosure controls and procedures are effective based on their evaluation of these controls and procedures as required by paragraph (b) of Exchange Act Rules 13a-15 or 15d-15.

#### **Changes in Internal Control over Financial Reporting**

We are in the process of implementing new enterprise resource planning software, Microsoft Dynamics D365 ("Dynamics"), as part of a plan to integrate and upgrade our systems and processes. The implementation of this software is scheduled to continue in phases over a number of years. During the second quarter of 2021, we completed the implementation of the financial reporting and consolidation modules. During the third quarter of 2021, we completed the implementation of the procurement modules. As the phased implementation of this system occurs, we expect certain changes to our processes and procedures which, in turn, will result in changes to our internal control over financial reporting. We expect Dynamics to strengthen our internal financial controls by automating a number of accounting and reporting processes and activities, thereby decreasing the number of manual processes previously required. Management will continue to evaluate and monitor our internal controls as processes and procedures in each of the affected areas evolve.

Other than as discussed above, no change in our internal control over financial reporting occurred during the quarter ended September 30, 2021 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

The information set forth under the heading “Legal Proceedings” in Note 6 of the Notes to Condensed Consolidated Financial Statements included in Item 1 of Part I of this Form 10-Q is incorporated by reference in response to this item.

#### Item 1A. Risk Factors.

##### Risks Related to Our Financial Position and Need for Additional Capital

*We have incurred net losses since inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability.*

Since inception, we have incurred recurring losses and negative cash flows from operations and, at September 30, 2021, we had an accumulated deficit of \$119.0 million. Our ability to achieve profitability depends on our ability to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize our products candidates. We do not anticipate generating revenues from product sales for the next year, if ever. We have devoted substantially all our efforts to date to research and development of our gene therapy product candidates as well as to building out our infrastructure. We expect that it could be at least a year, if ever, before we have a commercialized product candidate. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- continue our research and the clinical development of B-VEC, KB105, and KB301 including our current clinical trials and planned future trials;
- initiate clinical trials for KB104, KB407, KB408 and preclinical studies for any additional product candidates that we may pursue in the future;
- prepare our Biologics License Application (“BLA”), Marketing Authorisation Application and approvals in certain other countries for B-VEC;
- continue to operate our in-house commercial-scale cGMP manufacturing facility, ANCORIS, and complete the build out of our second cGMP manufacturing facility, ASTRA;
- manufacture material for clinical trials or potential commercial sales;
- further develop our gene therapy product candidate portfolio;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidate for which we may obtain marketing approval;
- develop, maintain, expand and protect our intellectual property portfolio;
- acquire or in-license other product candidates and technologies; and
- seek marketing approval for B-VEC and additional product candidates in the European Union (“EU”) and in other key geographies.

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, including completing the clinical trials for our product candidates, developing and validating commercial scale manufacturing processes, obtaining marketing approval for our product candidates, manufacturing, marketing and selling any product candidates for which we may obtain marketing approval and satisfying any post-marketing requirements. If we were required to discontinue development of any of our product candidates, if any of our product candidates do not receive regulatory approval, if we do not obtain our targeted indications for our product candidates or if any of our product candidates fails to achieve sufficient market acceptance for any indication, we could be delayed by many years in our ability to achieve profitability, if ever, and this would materially adversely affect our business prospects and financial condition. Moreover, if we decide to leverage any success with our B-VEC, KB105, KB301, KB104 or KB407 product candidates to develop other product opportunities, we may not be successful in such efforts, and our business could be materially adversely affected.



We currently have three product candidates, B-VEC, KB105, and KB301 in clinical trials and we may never develop, acquire or in-license additional product candidates. We may never succeed in any or all these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause investors to lose all or part of their investment.

Because of the numerous risks and uncertainties associated with pharmaceutical product and biological development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. If we are required by the FDA, the European Medicines Agency ("EMA"), or other regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of B-VEC, KB105, and KB301 our expenses could increase and revenue could be further delayed.

***We may need to raise additional funding in order to receive approval for our other product candidates. Such funding may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.***

To complete the process of obtaining regulatory approval for our product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our product candidates, if approved, we may require substantial additional funding. In addition, if we obtain marketing approval for our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. We anticipate that we may need additional funding to complete the development of B-VEC, KB105, KB301 and any future product candidates and to commercialize any such approved products.

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

- the progress, timing, results and costs of our Phase 3 clinical trials for B-VEC;
- the progress, timing, results and costs of our Phase 1/2 clinical trials for KB105;
- the progress, timing, results and costs of our Phase 1 clinical trials for KB301;
- the continued development and the filing of investigational new drug ("IND") applications for KB104, KB407, KB408 and other product candidates;
- the initiation, scope, progress, timing, costs and results of drug discovery, laboratory testing, manufacturing, preclinical studies and clinical trials for any other product candidates that we may pursue in the future, if any;
- the costs of building and maintaining our own commercial-scale cGMP manufacturing facilities;
- the outcome, timing and costs of seeking regulatory approvals;
- the costs associated with the manufacturing process development and evaluation of third-party manufacturers, if necessary;
- the extent to which the costs of our product candidates, if approved, will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors;
- the costs of commercialization activities for our current and future product candidates if we receive marketing approval for B-VEC, KB105, KB301 or any other product candidates we may develop, including the costs and timing of establishing product sales, medical affairs, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, if any, revenue received from commercial sale of our current and future product candidates;
- the terms and timing of any future collaborations, licensing, consulting or other arrangements that we may establish;
- the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, maintenance, defense and enforcement of any patents or other intellectual property rights, including milestone and royalty payments and patent prosecution fees that we are obligated to pay pursuant to our license agreements, if any;
- our current license agreements, if any, remaining in effect and our achievement of milestones under those agreements; and

- the extent to which we acquire or in-license other product candidates and technologies.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. Our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, will be derived from or based on sales of product candidates that may not 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. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and a portion of our operating cash flows, if any, being dedicated to the payment of principal and interest on such indebtedness, and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Furthermore, existing stockholders may not agree with our financing plans or the terms of such financings. Adequate additional financing may not be available to us on acceptable terms, or at all. The terms of additional financing may be impacted by, among other things, general market conditions, the market's perception of our product candidates and growth potential and the market price per share of our common stock

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

We are a development-stage company that commenced operations in 2016. Our efforts to date, with respect to the development of our product candidates have been limited to organizing and staffing our company, business planning, raising capital, developing our vector platform and related technologies, identifying potential gene therapy product candidates and undertaking preclinical studies and clinical trials of B-VEC, KB105, KB301, KB104, KB407 and KB408. While we have conducted clinical trials of B-VEC, KB105, and KB301, we have not yet demonstrated the ability to complete clinical trials of any of our product candidates, obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success, performance or viability may not be as accurate as they could be if we had more experience developing gene therapy products.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We will need to transition from a company with a research and development focus to a company capable of undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays and may not be successful in such a transition.

## **Risks Related to Our Business**

***Business interruptions resulting from the coronavirus disease 2019 ("COVID-19") outbreak or similar public health crises could cause a disruption of the development efforts for our product candidates and adversely impact our business.***

Public health crises such as pandemics or similar outbreaks could adversely impact our business. The COVID-19 pandemic is evolving, and to date has led to the implementation of various mitigation responses, including government-imposed quarantines, travel restrictions and other public health safety measures, as well as leading to reported adverse impacts on healthcare resources, facilities and providers across the United States and in other countries. The extent to which COVID-19 impacts our operations or those of our third-party partners will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the duration of the outbreak, additional or modified government actions, new information that will emerge concerning the severity and impact of COVID-19 and the actions to contain COVID-19 or address its impact in the short and long term, among others.

Additionally, timely initiation and completion of planned clinical trials is dependent upon the availability of, for example, clinical trial sites, researchers and investigators, regulatory agency personnel, and materials, which may be adversely affected by global health matters, such as pandemics. We plan to conduct clinical trials in geographies that are currently being affected by COVID-19.

In the event that we or governmental authorities were to modify or implement restrictions, our employees conducting research and development or manufacturing activities may not be able to access our laboratory or manufacturing spaces, and our core activities may be significantly limited or curtailed, possibly for an extended period of time.

Some factors from the COVID-19 pandemic that could delay or otherwise adversely affect the completion of our preclinical activities and the planned initiation of our clinical trials for our investigational drug product candidates, as well as our business operations generally, include:

- the potential diversion of healthcare resources away from the conduct of preclinical activities and clinical trials to focus on pandemic concerns, including the availability of necessary materials and the attention of physicians serving as our clinical trial investigators, hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our prospective clinical trials;
- limitations on travel that could interrupt key preclinical and clinical trial activities, such as clinical trial site initiations and monitoring, domestic and international travel by employees, contractors or patients to clinical trial sites, including any government-imposed travel restrictions, quarantines, or vaccine mandates that will impact the ability or willingness of patients, employees or contractors to travel to our research, manufacturing and clinical trial sites or secure visas or entry permissions, any of which could delay or adversely impact the conduct or progress of our prospective clinical trials;
- interruption or delays in the operations of the FDA and comparable foreign regulatory agencies, which may impact our ability to conduct preclinical and clinical activities as well as product approval timelines;
- limitations on our business operations by local, state, or the federal government that could impact our ability to conduct our preclinical or clinical activities, including completing our IND-enabling studies or our ability to select future development candidates; and interruption in global shipping affecting the transport of clinical trial materials, such as patient samples, investigational drug product candidates and conditioning drugs and other supplies used in our prospective clinical trials;
- interruption of, or delays in receiving, key materials from our suppliers and vendors due to staffing shortages, travel limitations, production slowdowns or stoppages and disruptions in delivery systems;
- interruption of, or delays in manufacturing our product candidates at our manufacturing facility in Pittsburgh or receiving supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, travel limitations, production slowdowns or stoppages and disruptions in delivery systems; and
- business disruptions caused by potential office, manufacturing and laboratory closures and an increased reliance on employees working from home, disruptions to or delays in ongoing laboratory experiments and operations, staffing shortages, travel limitations, cyber security and data accessibility, or communication or mass transit disruptions, any of which could adversely impact our business operations or delay necessary interactions with local regulators, ethics committees, manufacturing sites, research sites and other important agencies and contractors.

These and other factors arising from COVID-19 could adversely impact our ability to conduct clinical trials and our business generally and could have a material adverse impact on our operations and financial condition and results.

In addition, the trading prices for our common stock and other biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms. Further, conditions in the bank lending, capital and other financial markets may deteriorate as a result of the pandemic such that our access to capital and other sources of funding may be constrained.

The COVID-19 pandemic continues to evolve. The extent to which the outbreak may impact our business, preclinical studies and planned clinical trials will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the duration of the outbreak, travel restrictions and other actions to contain the outbreak or address its impact, such as social distancing and quarantines or lock-downs in the United States and other countries, business closures or business disruptions and the effectiveness of actions taken in the United States and other countries to contain and address the disease.

***We are a development-stage company. If we are unable to advance our product candidates through clinical trials, obtain regulatory approval and ultimately commercialize our product candidates, or if we experience significant delays in doing so, our business will be materially harmed.***

We are a development stage company, and B-VEC entered its first clinical trial in May 2018, KB105 entered its first clinical trial in September 2019, and KB301 entered its first trial in August 2020. The development and commercialization of our product candidates are subject to many uncertainties, including the following:

- successful enrollment and completion of clinical trials;
- positive results from our current and planned future clinical trials;
- receipt of regulatory approvals from applicable regulatory authorities;
- successful development of our internal manufacturing processes on an ongoing basis and maintenance of our existing arrangements with third-party manufacturers for clinical supply;
- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others; and
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;

If we fail in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.

***Our lead candidate, B-VEC, is still in clinical development, and there is no guarantee that the results from preclinical studies will be indicative of our ability to complete or the results to be obtained in the current Phase 3 clinical trials.***

We announced positive interim results from the Phase 1 portion of our Phase 1/2 clinical trial of B-VEC in October 2018 and positive interim results from the Phase 2 portion in June 2019, and complete Phase 1/2 results in May 2020. We commenced Phase 3 clinical trials for B-VEC in July 2020. There is no guarantee that results of this or any potential future clinical trials will be positive or that we will be able to complete this or any potential future clinical trials on the anticipated timelines, or at all. The positive results we have observed for B-VEC may not be predictive of the ultimate outcome of any future clinical trials, and the current and future clinical trial process may fail to demonstrate that B-VEC is safe for humans and effective for indicated uses, which may cause us to abandon B-VEC. Furthermore, research and discoveries by us or others may identify serious adverse events, undesirable side effects or other unexpected properties of our current and future product candidates, including B-VEC, that could delay, prevent or cause the withdrawal of regulatory approval, limit the commercial potential, or result in significant negative consequences following marketing approval.

***Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize B-VEC and the approval may be for a narrower indication than we seek.***

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if B-VEC meets its 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 or 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 post-approval safety monitoring program. 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 B-VEC. Any of the foregoing scenarios could materially harm the commercial prospects for B-VEC and materially and adversely affect our business, financial condition, results of operations and prospects.

***B-VEC is based on a novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval.***

The clinical trial requirements of the FDA, 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 such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied product candidates. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in

either the United States or the EU or how long it will take to commercialize our product candidates. Approvals by the European Commission may not be indicative of what the FDA may require for approval, and approval by the FDA may not be indicative of what the European Commission would require for approval.

Regulatory requirements and policy governing gene and cell therapy products have changed frequently and may continue to change in the future. The FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research (“CBER”) to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review. If we were to engage a National Institutes of Health funded institution to conduct a clinical trial, that institution’s Institutional Biosafety Committee as well as its Institutional Review Board (“IRB”), would need to review the proposed clinical trial to assess the safety of the trial. Similarly, 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.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of B-VEC or future product candidates or lead to significant post-approval limitations or restrictions. As we advance B-VEC, we may be required to consult with these regulatory and advisory groups and will need to comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of B-VEC. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects would be materially and adversely affected.

***B-VEC may cause undesirable side effects or have other properties that could delay or prevent its regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.***

There have been several significant adverse side effects in gene therapy trials using other vectors in the past. Gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. The risk of cancer remains a concern for gene therapy, and we cannot assure that it will not occur in any of our planned or future clinical studies. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur with B-VEC, our clinical trials could be suspended or terminated. If we are unable to demonstrate that such adverse events were caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, B-VEC for any or all targeted indications. Even if we can demonstrate that any 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 B-VEC, the commercial prospects may be harmed and our ability to generate product revenues may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Additionally, if B-VEC receives marketing approval, the FDA could require us to adopt a post-approval safety monitoring program to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by B-VEC, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of B-VEC;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way B-VEC is administered or conduct additional clinical trials;
- 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 B-VEC and could significantly harm our business, financial condition, results of operations and prospects.

***We may encounter substantial delays in our clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.***

Before obtaining marketing approval from regulatory authorities for the sale of our drug candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the drug candidate for its intended indications. Clinical trials are expensive, time consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in opening sites and recruiting suitable patients to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or concerns with a class of drug candidates, or after an inspection of our clinical trial operations or trial sites;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- occurrence of serious adverse events associated with the drug candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

In addition, if we make manufacturing or formulation changes to a product candidate, we may need to conduct additional studies to bridge our modified product candidate to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could limit our potential revenue or impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects. Any delays, setbacks or failures in our clinical trials could materially and adversely affect our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our drug candidates, we may:

- be delayed in obtaining marketing approval, if at all, or be required to conduct additional confirmatory safety and/or efficacy studies;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements;
- be required to perform additional clinical trials to support approval;
- have regulatory authorities withdraw, or suspend, their approval of the drug or impose restrictions on its distribution;
- be sued; or
- experience damage to our reputation.

Our drug development costs will also increase if we experience delays in testing or obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, need to be restructured or be completed on schedule, if at all.

Further, we, the FDA or an IRB, may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's current Good Clinical Practice regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our IND applications or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials. 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 our drug candidates could be negatively impacted, and our ability to generate revenues from our drug candidates may be delayed.

***We have a limited number of employees and limited corporate infrastructure and may experience difficulties in managing growth.***

We are a small company with a limited number of employees and corporate infrastructure. We have experienced a period of significant expansion in headcount and expect to experience significant expansion of our facilities, infrastructure and overhead as we develop our own manufacturing facilities and increase our research and development efforts. Future growth will impose significant added capital requirements, as well as added responsibilities on members of management, including the need to identify, recruit, maintain and integrate new personnel. Our future financial performance and our ability to compete effectively will depend, in part, on our ability to manage any future growth effectively.

***Even if we obtain regulatory approval for a product candidate, our product candidates will remain subject to regulatory oversight.***

Even if we obtain any regulatory approval for B-VEC, our lead product candidate, it will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for B-VEC may also be subject to a post-approval safety monitoring program, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. Our current and each of our proposed clinical trials for B-VEC includes a five-year, long-term follow-up phase, limited to confirmed data collection from annual visits with standard care physicians. The holder of an approved BLA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or a regulatory authority disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of B-VEC or any future product candidate, a regulatory authority may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

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

The FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of B-VEC or any future product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that

we may have obtained and we may not achieve or sustain profitability, which would materially and adversely affect our business, financial condition, results of operations and prospects.

***While we have obtained orphan drug designation for B-VEC, KB105 and KB407, it may not effectively protect us from competition, and we may be unable to obtain orphan drug designation for our future product candidates. If our competitors are able to obtain orphan drug exclusivity for products that constitute the same drug and treat the same indications as our product candidates before us, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.***

On November 2, 2017, the FDA granted orphan drug designation to our lead product candidate, B-VEC, for the treatment of DEB. On April 16, 2018, the European Commission granted the Orphan Medicinal Product Designation for B-VEC. On August 7, 2018, the FDA granted orphan drug designation to our second product candidate, KB105, currently in clinical development for treatment of patients with TGM1-deficient autosomal recessive congenital ichthyosis ARCI, and on October 10, 2019, the European Commission granted the Orphan Medicinal Product Designation for KB105. On August 17, 2020, the FDA granted orphan drug designation to our most recent product candidate, KB407, currently in preclinical development, for the treatment of cystic fibrosis. Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as having a patient population of fewer than 200,000 individuals 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 EU, the European Commission, upon a recommendation from the EMA's Committee for Orphan Medicinal Products, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the EU. Additionally, orphan designation is granted for products 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 EU would be sufficient to justify the necessary investment in developing the drug or biologic product.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we will be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the EU. The exclusivity period in the EU can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even though we have obtained orphan drug exclusivity for B-VEC, KB105 and KB407, that exclusivity may not effectively protect the product candidates from competition because different drugs can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the EU, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although like the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply enough quantities of orphan medicinal product.

***Breakthrough therapy designation, Regenerative Medicine Advanced Therapy ("RMAT") designation, Fast Track designation or Rare Pediatric Disease designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development, regulatory review or approval process, and it does not increase the likelihood that any of our product candidates will receive marketing approval in the United States.***



The FDA granted Fast Track designation in the United States for B-VEC on May 23, 2018 and for KB105 on October 24, 2019. In addition, B-VEC was granted RMAT by the FDA on June 21, 2019 and Priority Medicine (“PRIME”) by the EMA in March 2019. The receipt of any of these designations for a product candidate may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA and EMA procedures and does not assure ultimate approval by either the FDA or EMA.

A RMAT/PRIME therapy product candidate is defined as a product candidate that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease. Drugs designated as RMAT therapies by the FDA are eligible for accelerated approval and increased interaction and communication with the FDA designed to expedite the development and review process. If a drug, or biologic in our case, is intended for the treatment of a serious or life-threatening condition and the biologic demonstrates the potential to address unmet medical needs for this condition, the biologic sponsor may apply for FDA Fast Track designation. Even after having received Fast Track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Many biologics that have received Fast Track designation have failed to obtain approval.

A sponsor who receives an approval for a drug or biologic for a “rare pediatric disease” may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. We received the designation of “rare pediatric disease” for B-VEC in December 2016, for KB105 in August 2018, for KB104 in April 2019, and for KB407 in September 2020, which could qualify us to receive a Rare Pediatric Priority Review Voucher.

There is no assurance we will receive RMAT, PRIME or breakthrough therapy or Fast Track designations for any of our product candidates and the receipt of any of these designations for a product candidate may not result in a faster development process, review or approval and does not assure ultimate approval by the FDA. Further, even though we have received rare pediatric disease designation for B-VEC, KB105, KB104 and KB407, we may not experience a faster review or approval for a subsequent marketing application.

***We may expend our limited resources to pursue a 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.***

We have limited financial and managerial resources. 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.

***If we are not successful in discovering, developing and commercializing additional product candidates, our ability to expand our business and achieve our strategic objectives would be impaired.***

Although a substantial amount of our efforts focuses on the potential approval of B-VEC, KB105, KB301, KB104, KB407 and KB408 a key component our strategy is to discover, develop and potentially commercialize a portfolio of product candidates to treat orphan diseases and ultimately, non-orphan diseases. Identifying new product candidates requires substantial technical, financial and human resources, whether any product candidates are ultimately identified. Even if we identify product candidates that initially show promise, we may fail to successfully develop and commercialize such product candidates for many reasons, including the following:

- the research methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render our product candidates obsolete;
- product candidates we develop may nevertheless be covered by third parties’ patents or other exclusive rights;
- a product candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

If we are unsuccessful in identifying and developing additional product candidates, our potential for growth will be impaired.

***We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully market or commercialize our lead product candidate, B-VEC or any future product candidate.***

We are aware of several companies and institutions that are currently developing alternative autologous or palliative gene therapy approaches for DEB and cystic fibrosis. Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if 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 product candidate that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier 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 B-VEC or any future product candidate uneconomical or obsolete, and we may not be successful in marketing B-VEC or any future product candidate against competitors.

In the future, even if we commercialize a product candidate faster than our competitors, we could also face competition from lower cost biosimilars in the United States or in Europe.

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

### **Risks Related to Manufacturing**

***Delays in obtaining regulatory approvals of the process and facilities needed to manufacture our product candidates or disruptions in our manufacturing process may delay or disrupt our product development and commercialization efforts.***

Before we can begin to commercially manufacture our product candidates, whether in a third-party facility or in our own facilities, we must pass a pre-approval inspection of our manufacturing facilities by the FDA before our product candidates can obtain marketing approval. A manufacturing authorization must also be obtained from the appropriate EU regulatory authorities. The timeframe required for us to obtain such approvals is uncertain. To obtain approval, we will need to ensure that all our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any product candidate that we may develop.

In addition, the manufacturing process used to produce our product candidates is complex, novel and has not been validated for commercial use. In order to produce enough quantities of our product candidates for future clinical trials and initial U.S. commercial demand, we will need to increase the scale of our manufacturing process. The production of our product candidates requires processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours 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. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and that our product candidates are made strictly and consistently in compliance with the process. 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. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

***Although we have established our own manufacturing facility for our product candidates, we may need to utilize third parties to conduct our product manufacturing for the near future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily.***

Even if we obtain the validation from the FDA of our cGMP manufacturing facility, we intend to maintain third-party manufacturing capabilities in order to provide multiple sources of supply. In the event that these third-party manufacturers do not successfully carry out their contractual duties, meet expected deadlines or manufacture our product candidates in accordance with regulatory requirements or if there are disagreements between us and these third-party manufacturers, we will not be able to complete, or may be delayed in completing, the preclinical studies required to support future IND submissions of product candidates or the clinical trials required for approval of our product candidates. In such instances, we may need to locate an appropriate replacement third-party manufacturer, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense prior to the approval of our product candidates and would thereby have a material adverse effect on our business, financial condition, results of operations and prospects.

If we or our third-party manufacturer fails to comply with applicable cGMP regulations, the FDA and foreign regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be materially harmed.

***Any contamination in our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules.***

Given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our ability to produce our product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

Some of the raw materials 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 our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially and adversely affect our development timelines and our business, financial condition, results of operations and prospects.

#### **Risks Related to Commercialization of Our Product Candidates**

***If we are unable to expand our market development capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any product revenue.***

We currently have a small market development organization. To successfully commercialize our product candidates, if approved, we plan to expand our capabilities to promote market access and build awareness. To successfully commercialize any products that may result from our development programs, we will need to further expand our market development organization, either on our own or with a third party. The development of our own market development team will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaboration agreements regarding any of our product candidates with third parties to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded medical affairs, marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential products. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients or third-party payors, we will not be able to generate significant revenues from such product, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

***Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our gene therapy product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.***

Gene therapy remains a novel technology. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in trials using other vectors. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products or 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.

***If the market opportunities for our product candidates are smaller than we believe they are, our product revenues may be adversely impacted, and our business may suffer.***

We have mainly focused our research and product development efforts to date on B-VEC for DEB. Our understanding of both the number of people who have this disease, as well as the subset of people with this disease who have the potential to benefit from treatment with B-VEC, are based on estimates in published literature. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of this disease. The number of patients in the United States, the EU and elsewhere may turn out to be lower than expected or these patients may not be otherwise amenable to treatment with B-VEC or may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

Further, there are several factors that could contribute to making the actual number of patients who receive B-VEC less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment will likely diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell damage. Lastly, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes.

***The commercial success of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community.***

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our product candidates. Even with the requisite approvals from the FDA in the United States, the EMA in the EU and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of our product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of our product candidates over alternative treatments, if available;
- the cost of our product candidates relative to alternative treatments, if available;
- the clinical indications for which our product candidates are approved by the FDA or the EMA;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;

- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products and their ability to meet market demand;
- publicity concerning our product candidates or competing products and treatments;
- any restrictions on the use of our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

***Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates, if approved, which would adversely affect our revenue and results of operations.***

We expect that coverage and reimbursement of pharmaceuticals may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. Drug pricing by pharmaceutical companies recently has come under increased scrutiny and continues to be subject to intense political and public debate in the U.S. and abroad. Government and private third-party payors have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the U.S. Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payors, which may render our product candidates, if approved, not commercially viable or may adversely affect our anticipated future revenues and gross margins.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to the pricing of pharmaceutical drugs generally could restrict the amount that we are able to charge for our products, which would adversely affect our anticipated revenue and results of operations.

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

We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford our product candidates. Accordingly, sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

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

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or

are available only at limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and government payors develop their coverage and reimbursement policies. Currently, no gene therapy product has been approved for coverage and reimbursement by the Centers for Medicare & Medicaid Services (“CMS”), the agency responsible for administering the Medicare program. It is difficult to predict what CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these types of products. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union Member States. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. It also can take a significant amount of time after approval of a product to secure pricing and reimbursement for such product in many countries outside the United States. In general, the prices of medicines under such systems are substantially lower than in the United States. 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 products may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenues.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, such as average sales price, average manufacturer price, and actual acquisition cost. The existing data for reimbursement based on some of these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. Therefore, it may be difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover candidate products that we are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional potential legislative and administrative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours.

***Ethical, legal and social issues related to genetic testing may reduce demand for our product candidates, if approved.***

We anticipate that prior to receiving certain gene therapies, patients may be required to undergo genetic testing. Genetic testing has raised concerns regarding the appropriate utilization and the confidentiality of information provided by genetic testing. Genetic tests for assessing a person’s likelihood of developing a chronic disease have focused public attention on the need to protect the privacy of genetic information. For example, concerns have been expressed that insurance carriers and employers may use these tests to discriminate based on genetic information, resulting in barriers to the acceptance of genetic tests by consumers. Concerns have also been raised about the accuracy of genetic testing. This could lead to governmental authorities restricting genetic testing or calling for additional regulation of genetic testing, particularly for diseases for which there is no known cure. Any of these scenarios could decrease demand for our product candidates, if approved.

***Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for them outside of the United States, which would limit our market opportunities and adversely affect our business.***

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of B-VEC or other future product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval.

Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our product candidates, if approved, is also subject to approval. We intend to submit a marketing authorization application to the EMA for approval of B-VEC in the EU but obtaining such approval from the European Commission following the opinion of the EMA is a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the EU also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be adversely affected.

***Increasing demand for compassionate use or expanded access of our unapproved therapies could negatively affect our reputation and harm our business.***

We are developing our product candidates for illnesses for which there are currently limited to no available therapeutic options. At least one other company has been the target of disruptive social media campaigns related to a request for access to unapproved drugs for patients with significant unmet medical need. If we experience a similar social media campaign regarding our decision to provide or not provide our product candidates under an expanded access corporate policy, our reputation may be negatively affected and our business may be harmed. Recent media attention to individual patients' expanded access requests has resulted in the introduction of legislation at the local and national level referred to as "Right to Try" laws, such as the Right to Try Act, which are intended to give patients access to unapproved therapies. New and emerging legislation regarding expanded access to unapproved drugs for life-threatening illnesses could negatively impact our business in the future.

A possible consequence of both activism and legislation in this area is the need for us to initiate an unanticipated expanded access program or to make our product candidates more widely available sooner than anticipated. We are a small company with limited resources and unanticipated trials or access programs could result in diversion of resources from our primary goals.

In addition, some patients who receive access to unapproved drugs through compassionate use or expanded access programs have life-threatening illnesses and have exhausted all other available therapies. The risk for serious adverse events in this patient population is high which could have a negative impact on the safety profile of our product candidates if we were to provide them to these patients in accordance with our expanded access corporate policy, which could cause significant delays or an inability to successfully commercialize our product candidates, which could materially harm our business. If we were to provide patients with our product candidates under our expanded access corporate policy, we may in the future need to restructure or pause ongoing compassionate use and/or expanded access programs in order to perform the controlled clinical trials required for regulatory approval and successful commercialization of our product candidates, which could prompt adverse publicity or other disruptions related to current or potential participants in such programs

**Risks Related to Our Business Operations**

***We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates that may be a greater commercial opportunity or for which there is a greater likelihood of success.***

The success of our business depends upon our ability to identify, develop and commercialize product candidates based on our gene therapy platform. Research programs to identify new product candidates require substantial technical, financial and human resources. Although certain of our product candidates are currently in clinical or preclinical development, we may fail to identify other potential product candidates for clinical development for several reasons. For example, our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

***If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.***

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain enough numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

***Our future success depends on our ability to retain key employees, including scientific and technical personnel, and to attract, retain and motivate qualified personnel.***

We are highly dependent on members of our management team, the loss of whose services may adversely impact the achievement of our objectives. Our employees are at-will employees, and the loss of one or more of them might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining other qualified employees for our business, including scientific and technical personnel, also will be critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, key employees or advisors, may impede the progress of our research, development and commercialization objectives and have a material adverse effect on our business, financial condition, results of operations and prospects.

***Our employees, principal investigators and advisors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.***

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



condition, results of operations and prospects, including the imposition of significant fines, criminal penalties, or other sanctions.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of our current and future drug candidates.

***Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.***

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval.

In the U.S., there have been and continue to be a number of legislative efforts to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (“PPACA”), was passed, which substantially changes the way healthcare is financed by both the government and private insurers, and significantly impacts the U.S. pharmaceutical industry. The PPACA, among other things: (i) addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; (ii) increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations; (iii) establishes annual fees and taxes on manufacturers of certain branded prescription drugs; (iv) expands the availability of lower pricing under the 340B drug pricing program by adding new entities to the program; and (v) establishes a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer’s outpatient drugs to be covered under Medicare Part D.

Since its enactment, there have been numerous judicial, administrative, executive, and legislative challenges to certain aspects of the PPACA, and we expect there will be additional challenges in the future. As a result, there have been delays in the implementation of, and action taken to attempt to repeal or replace, certain aspects of the PPACA. Litigation and legislation over the ACA may continue in the future, with unpredictable and uncertain results.

We cannot predict the impact that such actions against the ACA or other health care reform efforts in the future will have on our business, and there is uncertainty as to what healthcare programs and regulations may be implemented or changed at the federal and/or state level in the United States, or the effect of any future legislation or regulation. However, it is possible that such initiatives could have an adverse effect on our ability to obtain approval and/or successfully commercialize products in the United States in the future. For example, any changes that reduce, or impede the ability to obtain, reimbursement for the type of products we intend to commercialize in the United States (or our products more specifically, if approved) could adversely affect our business plan to introduce our products in the United States.

Other legislative changes have been proposed and adopted in the United States since the PPACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation’s automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2027 unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to certain providers, and increased the time for Medicare contractors to recoup Medicare overpayments to providers from three to five years.

Further, there has been heightened governmental scrutiny in recent years over the manner in which manufacturers set prices for their marketed products and the cost of prescription drugs to consumers and government healthcare programs, which have resulted in several recent Congressional inquiries and proposed and enacted bills designed to, among other things, reduce the cost of prescription drugs, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, the

United States government, state legislatures, and foreign governments have shown significant interest in implementing cost containment programs, including price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs to limit the growth of government paid health care costs. For example, the United States government has passed legislation requiring pharmaceutical manufacturers to provide rebates and discounts to certain entities and governmental payors to participate in federal healthcare programs. Further, Congress and the current administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs, and the current administration recently released a “Blueprint”, or plan, to reduce the cost of drugs. The current administration’s Blueprint contains certain measures that the US Department of Health and Human Services is already working to implement. Individual states in the United States have also been increasingly passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Additional changes may affect our business, including those governing enrollment in federal healthcare programs, reimbursement changes, fraud and abuse enforcement, and expansion of new programs, such as Medicare payment for performance initiatives.

These initiatives, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms could result in reduced demand for our product candidates or additional pricing pressures and may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

***We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.***

If we obtain FDA approval for our product candidates and begin commercializing in the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Anti-Kickback Statute, federal civil and criminal false claims laws and the Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business as well as other jurisdictions. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. The PPACA amended the intent requirement of the federal Anti-Kickback Statute to clarify that a person or entity does not have to have actual knowledge of this statute or specific intent to violate it;
- federal civil and criminal false claims laws and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. The PPACA provides that a claim for items or services resulting from an Anti-Kickback Statute violation is a false claim under the federal False Claims Act. Cases against pharmaceutical manufacturers support the view that certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”) which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (“HITECH”), and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach;

- Notification Rules under HITECH and the Genetic Information Nondiscrimination Act; Other modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;
- federal transparency laws, including the federal Physician Payment Sunshine Act, that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to report annually to the CMS information related to: (i) payments or other “transfers of value” made to physicians and teaching hospitals and (ii) ownership and investment interests held by physicians and their immediate family members;
- state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states; and
- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Often, to avoid the threat of treble damages and penalties under the False Claims Act, health care providers will resolve allegations in a settlement without admitting liability. Any such settlement could materially affect our business, financial operations, and reputation.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management’s attention from the operation of our business. The shifting compliance environment and the need to build and maintain a robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic materials. Our operations also produce hazardous waste products. 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. We also could incur significant costs associated with civil or criminal fines and penalties. 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.

Although we maintain workers’ compensation insurance for certain costs and expenses, we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate

coverage against potential liabilities. We do not maintain insurance for toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials.

We also may incur substantial costs to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could materially adversely affect our business, financial condition, results of operations and prospects.

***Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.***

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets, including conditions that are outside of our control, such as the U.S. presidential election and the impact of health and safety concerns, such as the current coronavirus outbreak. The most recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the most recent global financial crisis, could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our suppliers, possibly resulting in supply disruption, or cause delays in payments by third-party payors or our collaborators. Any of the foregoing could harm our business and we cannot anticipate all the ways in which the current economic climate and financial market conditions could adversely impact our business.

***Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.***

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical 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 confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed.

***Cyber-security incidents, including data security breaches or computer viruses, could harm our business by disrupting our delivery of services, damaging our reputation or exposing us to liability.***

We receive, process, store, and transmit, often electronically, confidential data of others. Unauthorized access to our computer systems or stored data could result in the theft or improper disclosure of confidential information, the deletion or modification of records, or could cause interruptions in our operations. These cyber-security risks increase when we transmit information from one location to another, including transmissions over the Internet or other electronic networks. Despite implemented security measures, our facilities, systems, and procedures, and those of our third-party service providers, may be vulnerable to security breaches, acts of vandalism, software viruses, misplaced or lost data, programming and/or human errors, or other similar events which may disrupt our delivery of services or expose the confidential information of our customers and others. Any security breach involving the misappropriation, loss or other unauthorized disclosure or use of confidential information of others, whether by us or a third party, could: (i) subject us to civil and criminal penalties; (ii) have a negative impact on our reputation; or (iii) expose us to liability to our customers, third parties or government authorities.

***Our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.***

Natural disasters could severely disrupt our operations or the operations of manufacturing facilities and have a material adverse effect on our business, financial condition, results of operations and prospects. 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 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 that we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. Substantially all our current supply of our product candidates is located at our manufacturing facility in Pittsburgh, Pennsylvania. We are constructing an additional manufacturing facility and establishing a relationship with a third-party contract manufacturer as a back-up supplier.

for the commercial supply of our products, if necessary. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

### **Risks Related to Our Intellectual Property**

***If we are unable to obtain and maintain adequate U.S. and foreign patent protection for our product candidates, including B-VEC, KB105, KB301, KB104, KB407, KB408 any future product candidates we may develop, and/or our vector platform, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technologies similar or identical to ours, and our ability to successfully commercialize our current product candidates, any future product candidates we may develop, and our platform technologies 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 B-VEC, KB105, KB301, KB104, KB407, KB408 and additional product candidates in our pipeline, current and future innovations related to our vector platform, and our institutional knowledge. The patent prosecution process is expensive, time-consuming and complex; we may not be able to file, prosecute, maintain, and/or enforce all necessary or desirable patent applications and issued patents at a reasonable cost or in a timely manner. We currently have six issued patents in the United States: (1) U.S. patent No. 9,877,990, covering, in part, pharmaceutical formulations comprising our lead clinical product B-VEC, as well as methods of its use for treating wounds, disorders, and diseases of the skin, which we refer to as the '990 patent; (2) U.S. patent No. 10,155,016 covering pharmaceutical compositions containing B-VEC formulated for myriad routes of administration; (3) U.S. patent No. 10,441,614 covering aspects of our vector platform technology, and its uses in delivering any gene of interest to the skin; (4) U.S. patent No. 10,525,090, covering pharmaceutical compositions comprising our second clinical product candidate, KB105, and methods of its use for treating TGM1-deficient autosomal recessive congenital ichthyosis; (5) U.S. Patent No. 10,786,438 covering pharmaceutical compositions comprising vectors encoding cosmetic proteins, including our third product candidate, KB301, and methods for their use for improving skin condition, quality, and/or appearance; and (6) U.S. Patent No. 10,829,529 covering the methods of using KB407 for the treatment of cystic fibrosis and other diseases causing progressive lung destruction. Furthermore, we have international patent applications filed in accordance with the Paris Cooperation treaty directed to multiple discovery, preclinical, and clinical programs, including B-VEC, KB105, KB301, KB104, KB407, and KB-408, as well as multiple patent applications filed in foreign jurisdictions stemming from these international applications. B-VEC is also the subject of multiple patents granted in foreign jurisdictions, including European Patent No. 3 377 637 B1, covering pharmaceutical compositions containing B-VEC as well as uses thereof.

Even if we are granted the patents that we are currently pursuing, they may not issue in a form that will provide us with the full scope of protection we desire, they may not prevent competitors or other third parties from competing with us, and/or they may not otherwise provide us with a competitive advantage. 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. For example, there is no assurance that the '990 patent, or any other patent we are granted, will prevent third parties from developing competing technologies. Moreover, our patent estate, including the '990 patent, does not preclude third parties from having intellectual property rights that could interfere with our freedom to use our platform, including for dermatological indications. Even assuming patents issue from our pending and future patent applications, changes in either the patent laws or interpretation of the patent laws in the United States and foreign jurisdictions may diminish the value of our patents or narrow their scope of protection.

We also may not be aware of all third-party intellectual property rights potentially relating to technologies similar to our own. Publications of discoveries in the scientific literature often lag their 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, it is impossible to be certain that we were the first to develop the specific technologies as claimed in any owned patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

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

Filing, prosecuting and defending patents on each and every one of our product candidates, current and future innovations related to our vector platform, and our institutional knowledge in all countries throughout the world would be prohibitively expensive, and intellectual property rights in some countries outside the United States may differ in scope from those eventually granted in the United States. Thus, in some cases, we may not have the opportunity to obtain patent protection for certain technologies in some jurisdictions outside the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, even in jurisdictions where we

do pursue patent protection. Competitors may use our technologies in jurisdictions where we have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates, 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 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. Such challenges in enforcing rights in these countries could make it difficult for us to stop the infringement of our patents, if pursued and obtained, or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our current and future patent rights in foreign jurisdictions could result in substantial costs and may divert our efforts and attention from other aspects of our business; could put our patents at risk of being invalidated or interpreted narrowly; could put any future patent applications, including continuation and divisional 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. Accordingly, our efforts to enforce any intellectual property rights around the world stemming from intellectual property that we develop may be inadequate to obtain a significant commercial advantage in these foreign jurisdictions.

***Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.***

Our commercial success depends upon our ability (and the ability of any potential future collaborators) to develop, manufacture, market and sell our product candidates, and to freely use our proprietary technologies (*e.g.*, without infringing the rights and intellectual property of others). Many companies and institutions have filed, and continue to file, patent applications related to various aspects of gene therapy. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing, and can be revised before issuance, there may be applications now pending which may later result in issued patents that a third-party asserts are infringed by the manufacture, use, sale, or importation of our products. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. On May 1, 2020, a complaint was filed against us in the United States District Court for the Western District of Pennsylvania by PeriphaGen Inc., which also named our Chief Executive Officer and Chief Operating Officer, Krish Krishnan and Suma Krishnan, respectively. The complaint alleges breach of contract and misappropriation of trade secrets, which secrets the plaintiff asserts were used to develop our product candidates, including the vector backbones, and our STAR-D platform. For more information, see "Legal Proceedings" in Note 6 of the Notes to Condensed Consolidated Financial Statements included in Item 1 of Part I of this Form 10-Q. We may in the future become party to, or be threatened with, other adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates or related technologies, including, for example, interference proceedings, post grant review challenges, and *inter partes* review before the United States Patent and Trademark Office ("USPTO"). For example, a third party may bring an *inter partes* review challenging our patents and any future patent that may be granted to us. Our competitors or other third parties may assert infringement claims against us, alleging that our therapeutics, manufacturing methods, formulations or administration methods are covered by their patents. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue, and against whom our patent portfolio may therefore have no deterrent effect.

There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patents or other intellectual property rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could materially and adversely affect our ability to commercialize our products, including B-VEC. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. In such a hypothetical situation, there is no assurance that a court of competent jurisdiction would find that our product candidates or technologies do not infringe a third-party patent.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcomes are uncertain. If we are found, or believe there is a risk that we may be found, to infringe a third party's valid and enforceable intellectual property rights, we could be required (or may choose) to obtain a license from such a third party to continue developing, manufacturing and marketing our technologies. However, we may not be able to obtain any required license on commercially reasonable terms, if 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 further, it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing

and commercializing the infringing technologies, including B-VEC. We also could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our technologies, including B-VEC, or force us to cease some or all our business operations. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

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

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming. Competitors may infringe our current or future patents, should such patents issue, or we may be required to defend against claims of infringement or other unauthorized use of intellectual property. 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 scientific and management personnel from their normal responsibilities. 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. In addition, there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing, or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing, misappropriating, or successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

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

Certain of our employees or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including potential competitors, and we have and may in the future enter into agreements providing us with rights to intellectual property of third parties for limited purposes. Although we try to observe the terms of agreements under which we obtain access to third party intellectual property and to ensure that our employees and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals, or we, have used or disclosed intellectual property, including trade secrets or other proprietary information, of third parties or the current or former employers of employees or advisors. For instance, as described in Note 6 of the Notes to Condensed Consolidated Financial Statements included in Item 1 of Part I of the Form 10Q on May 1, 2020, a complaint was filed against us by PeriphaGen Inc., which also named our Chief Executive Officer and our Chief Operating Officer, Krish Krishnan and Suma Krishnan, respectively. The complaint alleges breach of contract and misappropriation of trade secrets, which secrets the plaintiff asserts we used to develop our vector platform and product candidates. If we fail in defending any such claims, in addition to paying monetary damages, we may be subject to an injunction and may lose valuable intellectual property rights or personnel. Moreover, any such litigation, or the threat thereof, may adversely affect our ability to hire new employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize our technologies, which would have an adverse effect on our business, results of operations, and financial condition. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

While it is our policy to require our employees and contractors who may be involved in the conception of intellectual property to execute agreements assigning such intellectual property rights to us, unforeseen complications may arise when fully and adequately executing such an agreement with each party who, in fact, conceives of intellectual property that we regard as our own. Examples of such complications may include, for example, when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached. Such complications may lead to us being forced to bring claims against third parties or current and former employees, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Moreover, individuals executing agreements with us may have preexisting or competing obligations to a third party, such as an academic

institution, and thus an agreement with us may be insufficient in fully perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property that we may own may have a material adverse effect on our business.

***Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.***

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. For example, on September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act included several significant changes to U.S. patent law, including provisions that affected the way patent applications are prosecuted, and altered strategies regarding patent litigation. These provisions also switched the United States from a “first-to-invent” system to a “first-to-file” system, allowed third-party submissions of prior art to the USPTO during patent prosecution, and set forth additional procedures to attack the validity of a patent through various post grant proceedings administered by the USPTO. As patent reform legislation can inject serious uncertainty into the patent prosecution and litigation processes, it is not clear what impact future patent reform legislation will have on the operation of our business. However, such future legislation, and its implementation, could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of any issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Moreover, the patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain given the ever evolving and constantly shifting nature of precedential patent cases decided by both the U.S. Court of Appeals for the Federal Circuit and the U.S. Supreme Court. We cannot assure you that our efforts to seek patent protection for our technology and product candidates will not be negatively impacted by the future court decisions or changes in guidance or procedures issued by the USPTO. These decisions, and any guidance issued by the USPTO (or changes thereto), could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property rights in the future.

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

We are in the process of registering certain of our trademarks and trade names. Once trademarks or trade names have been registered, they may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which are important for building name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. There also could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trade names that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to patents, trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

***Intellectual property rights and regulatory exclusivity rights do not necessarily address all potential threats.***

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

- others may be able to make gene therapy products that are similar to our product candidates but that are not covered by the claims of our current patents, or of patents that we may own or license in the future;
- we, or any future license partners or collaborators, might not have been the first to file patent applications covering certain aspects of the concerned technologies;
- others may independently develop similar or alternative technologies, or duplicate any of our technologies, potentially without falling within the scope of our current or future issued claims, thus not infringing our intellectual property rights;
- it is possible that our filed or future patent applications will not lead to issued patents;



- issued patents to which we currently hold rights or to which we may hold rights in the future may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- others may have access to any future intellectual property rights licensed to us on a non-exclusive basis;
- our competitors might conduct research and development activities in countries where we do not have or pursue 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 or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent application covering certain of our trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

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

### **Risks Related to Ownership of Our Common Stock**

***Our Chief Executive Officer and Chairman of the Board of Directors and our founder, Chief Operating Officer and director will have the ability to substantially influence all matters submitted to stockholders for approval.***

As of September 30, 2021, Krish S. Krishnan and Suma M. Krishnan, our Chief Executive Officer and Chairman of the Board and our founder, Chief Operating Officer and director, respectively, in the aggregate, beneficially owned shares representing approximately 17% of our capital stock. As a result, they will be able to substantially influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons would substantially influence the election of directors and approval of any merger, consolidation or sale of all or substantially all our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in management of our company that our public stockholders disagree with.

***If securities analysts publish negative evaluations of our stock, the price of our stock could decline.***

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

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

Our stock price has been and is likely to continue to be volatile. The stock market in general and the market for biopharmaceutical or pharmaceutical companies specifically has experienced extreme volatility that has often been unrelated to the operating performance of such companies. As a result of this volatility, you may not be able to sell your common stock at or above the price that you paid for it. The market price of our common stock may be influenced by many factors, including:

- our ability to successfully proceed to and conduct clinical trials;
- results of clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the United States and other countries;
- 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;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;

- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- our inability to obtain or delays in obtaining adequate product supply for any approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including intellectual property or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

***We will continue to incur costs as a result of becoming a public company, and such costs will increase when we cease to be an “emerging growth company.”***

As a public company, we expect to continue to incur significant legal, accounting, insurance and other expenses, including costs associated with public company reporting requirements. The expenses incurred by public companies generally for reporting and corporate governance purposes have been increasing. We expect compliance with these public reporting requirements and associated rules and regulations to increase expenses, particularly after we are no longer an emerging growth company beginning in 2022, although we are currently unable to estimate these costs with any degree of certainty. We will be an emerging growth company until the end of 2021, after which, we will incur additional costs applicable to public companies that are not emerging growth companies.

***If we fail to maintain effective internal control over financial reporting, we may not be able to accurately report our financial results, which may adversely affect investor confidence in our company and, as a result, the value of our common stock.***

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Beginning with our fiscal year ending December 31, 2022, we will cease to be an “emerging growth company” as defined by the JOBS Act. After we are no longer an emerging growth company under the JOBS Act, Section 404 of the Sarbanes-Oxley Act requires our auditors to deliver an attestation report on the effectiveness of our internal control over financial reporting in conjunction with their opinion on our audited financial statements. Substantial work on our part is required to implement appropriate processes, document the system of internal control over key processes, assess their design, remediate any deficiencies identified and test their operation. This process is expected to be both costly and challenging. We cannot give any assurances that material weaknesses will not be identified in the future in connection with our compliance with the provisions of Section 404 of the Sarbanes-Oxley Act. The existence of any material weakness would preclude a conclusion by management and our independent auditors that we maintained effective internal control over financial reporting. Our management may be required to devote significant time and expense to remediate any material weaknesses that may be discovered and may not be able to remediate any material weakness in a timely manner. The existence of any material weakness in our internal control over financial reporting could also result in errors in our financial statements that could require us to restate our financial statements, cause us to fail to meet our reporting obligations and cause investors to lose confidence in our reported financial information, all of which could lead to a decline in the per-share trading price of our common stock.

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

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called “poison pill,” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 80% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our bylaws.

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

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

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

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

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

***Third-party expectations relating to environmental, social and governance factors may impose additional costs and expose us to new risks.***

There is an increasing focus from certain investors and other stakeholders concerning corporate responsibility, specifically related to environmental, social and governance factors. Some investors may use these factors to guide their investment strategies and, in some cases, may choose not to invest in us if they believe our policies relating to corporate responsibility are inadequate. Third-party providers of corporate responsibility ratings and reports on companies have increased in number, resulting in varied and in some cases inconsistent standards. In addition, the criteria by which companies’ corporate responsibility practices are assessed are evolving, which could result in greater expectations of us and cause us to undertake costly initiatives to satisfy such new criteria. Alternatively, if we elect not to or are unable to satisfy such new criteria or do not meet the criteria of a specific third-party provider, some investors may conclude that our policies with respect to corporate responsibility are inadequate. We may face reputational damage in the event that our corporate responsibility procedures or standards do not meet the standards set by various constituencies. Furthermore, if our competitors’ corporate responsibility performance is perceived to be greater than ours, potential or current investors may elect to invest with our competitors instead. In addition, in the event that we communicate certain initiatives and goals regarding environmental, social and governance matters, we could fail, or be perceived to fail, in our achievement of such initiatives or goals, or we could be criticized for the scope of such initiatives or goals. If we fail to satisfy the expectations of investors and other stakeholders or our initiatives are not executed as planned, our reputation and financial results could be adversely affected.

**ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS**

**Sales of Unregistered Securities**

None.

**ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None.

**ITEM 4. MINE SAFETY DISCLOSURES**

Not applicable.

**ITEM 5. OTHER INFORMATION**

None.

**ITEM 6. EXHIBITS**

Exhibit Number	
31.1	<a href="#"><u>Certification of Periodic Report by Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</u></a>
31.2	<a href="#"><u>Certification of Periodic Report by Chief Accounting Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</u></a>
32.1	<a href="#"><u>Certification of Chief Executive Officer and Chief Accounting Officer Pursuant to 18 U.S.C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u></a>
101	Inline XBRL (Extensible Business Reporting Language). The following materials from this Quarterly Report on Form 10-Q for the period ended September 30, 2021, formatted in Inline XBRL: (i) consolidated balance sheets of Krystal Biotech, Inc., (ii) consolidated statements of operations of Krystal Biotech, Inc., (iii) consolidated statements of comprehensive income/(loss) of Krystal Biotech, Inc., (iv) consolidated statements of changes in equity of Krystal Biotech, Inc., (v) consolidated statements of cash flows of Krystal Biotech, Inc. and (vi) notes to consolidated financial statements of Krystal Biotech, Inc. The instance document does not appear in the interactive data file because its XBRL tags are embedded within the Inline XBRL document
104	Cover Page Interactive Data File - the cover page XBRL tags are embedded within the Inline XBRL

## 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.

KRYSTAL BIOTECH, INC.  
(Registrant)

Date: November 8, 2021

By: /s/ Krish S. Krishnan

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Krish S. Krishnan  
President and Chief Executive Officer  
(Principal executive officer)

By: /s/ Kathryn A. Romano

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Kathryn A. Romano  
Chief Accounting Officer  
(Principal financial and accounting officer)

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

I, Krish S. Krishnan, certify that:

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

Date: November 8, 2021

By: /s/ Krish S. Krishnan  
Krish S. Krishnan  
President and Chief Executive Officer

**CERTIFICATION OF CHIEF ACCOUNTING OFFICER  
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Kathryn A. Romano, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Krystal Biotech, 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 Condensed financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - c. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 8, 2021

By: /s/ Kathryn A. Romano  
Kathryn A. Romano  
Chief Accounting Officer



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

I, Krish S. Krishnan, Chief Executive Officer, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. the Quarterly Report on Form 10-Q for the three months ended September 30, 2021, (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of Krystal Biotech, Inc.

Date: November 8, 2021

By: /s/ Krish S. Krishnan  
Krish S. Krishnan  
President and Chief Executive Officer

I, Kathryn A. Romano, Chief Accounting Officer, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1. the Quarterly Report on Form 10-Q for the three months ended September 30, 2021, (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of Krystal Biotech, Inc.

Date: November 8, 2021

By: /s/ Kathryn A. Romano  
Kathryn A. Romano  
Chief Accounting Officer