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, 2020
or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from_____ to_____
Commission file number: 001-38210

Krystal Biotech, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**82-1080209**
(State or other jurisdiction of incorporation or organization)	**(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 30, 2020, there were 19,707,620 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

(In thousands, except shares and per share data)	(unaudited) September 30, 2020	December 31, 2019
Assets		
Current assets		
Cash and cash equivalents	$ 282,369	$ 187,514
Short-term investments	3,996	6,171
Prepaid expenses and other current assets	1,287	2,195
Total current assets	287,652	195,880
Property and equipment, net	17,512	8,475
Long-term investments	—	497
Prepaid rent	2,400	—
Right-of-use asset	2,476	2,709
Other non-current assets	1,411	1,462
Total assets	$ 311,451	$ 209,023
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 2,591	$ 1,021
Current portion of lease liability	564	480
Accrued expenses and other current liabilities	4,043	1,826
Total current liabilities	7,198	3,327
Lease liability	2,533	2,782
Total liabilities	9,731	6,109
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred stock; $0.00001 par value; 20,000,000 shares authorized at September 30, 2020 (unaudited) and December 31, 2019; 2,061,773 shares issued, and no shares outstanding at September 30, 2020 (unaudited) and December 31, 2019	—	—
Common stock; $0.00001 par value; 80,000,000 shares authorized at September 30, 2020 (unaudited) and December 31, 2019; 19,706,870 and 17,354,310 shares issued and outstanding at September 30, 2020 (unaudited) and December 31, 2019, respectively	—	—
Additional paid-in capital	362,531	241,951
Accumulated other comprehensive income	20	10
Accumulated deficit	(60,831)	(39,047)
Total stockholders' equity	301,720	202,914
Total liabilities and stockholders' equity	$ 311,451	$ 209,023

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, 2020	Three Months Ended September 30, 2019	Nine Months Ended September 30, 2020	Nine Months Ended September 30, 2019
Expenses				
Research and development	$ 5,100	$ 3,885	$ 12,264	$ 11,267
General and administrative	4,580	1,457	10,315	4,660
Total operating expenses	9,680	5,342	22,579	15,927
Loss from operations	(9,680)	(5,342)	(22,579)	(15,927)
Other Income				
Interest and other income, net	70	1,070	795	2,196
Net loss	(9,610)	(4,272)	(21,784)	(13,731)
Unrealized gain (loss) on available-for-sale securities	(20)	(7)	10	9
Comprehensive loss	$ (9,630)	$ (4,279)	$ (21,774)	$ (13,722)
Net loss per common share: Basic and diluted	$ (0.49)	$ (0.25)	$ (1.18)	$ (0.89)
Weighted-average common shares outstanding: Basic and diluted	19,676,016	17,291,245	18,477,495	15,420,995

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 Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
Balances at January 1, 2020	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)
Balances at March 31, 2020	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)
Balances at June 30, 2020	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)
Balances at September 30, 2020	19,706,870	$ —	$ 362,531	$ 20	$ (60,831)	$ 301,720

(In thousands, except shares)	Common Stock Shares	Common Stock Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
Balances at January 1, 2019	14,428,916	$ —	$ 133,183	$ 2	$ (19,959)	$ 113,226
Issuance of common stock, net	14,653	—	44	—	—	44
Stock-based compensation expense	—	—	313	—	—	313
Unrealized gain on investments	—	—	—	14	—	14
Net loss	—	—	—	—	(4,111)	(4,111)
Balances at March 31, 2019	14,443,569	$ —	$ 133,540	$ 16	$ (24,070)	$ 109,486
Issuance of common stock, net	2,501,500	—	93,825	—	—	93,825
Stock-based compensation expense	—	—	325	—	—	325
Unrealized gain on investments	—	—	—	2	—	2
Net loss	—	—	—	—	(5,348)	(5,348)
Balances at June 30, 2019	16,945,069	$ —	$ 227,690	$ 18	$ (29,418)	$ 198,290
Issuance of common stock, net	362,571	—	13,357	—	—	13,357
Stock-based compensation expense	—	—	278	—	—	278
Unrealized loss on investments	—	—	—	(7)	—	(7)
Net loss	—	—	—	—	(4,272)	(4,272)
Balances at September 30, 2019	17,307,640	$ —	$ 241,325	$ 11	$ (33,690)	$ 207,646

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, 2020	2019
Operating Activities		
Net loss	$ (21,784)	$ (13,731)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation and amortization	1,361	753
Stock-based compensation expense	2,702	916
Loss on disposals of fixed assets	33	54
Changes in operating assets and liabilities		
Prepaid expenses and other current assets	489	(465)
Prepaid rent	(2,400)	—
Lease liability	(165)	(55)
Accounts payable	725	170
Accrued expenses and other current liabilities	980	668
Net cash used in operating activities	(18,059)	(11,690)
Investing Activities		
Purchases of property and equipment	(7,636)	(4,120)
Purchases of short-term investments	(3,205)	(6,867)
Proceeds from maturities of short-term investments	5,877	6,587
Net cash used in investing activities	(4,964)	(4,400)
Financing Activities		
Issuance of common stock, net	117,878	107,226
Net cash provided by financing activities	117,878	107,226
Net increase in cash and cash equivalents	94,855	91,136
Cash and cash equivalents at beginning of period	187,514	103,670
Cash and cash equivalents at end of period	$ 282,369	$ 194,806
Supplemental Disclosures of Non-Cash Investing and Financing Activities		
Unpaid purchases of property and equipment	$ 3,173	$ 1,026
Initial recognition of right-of-use assets	$ —	$ 3,066

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

1. Organization

Krystal Biotech, Inc. and its consolidated subsidiary (the "Company," or "we" or other similar pronouns) commenced operations in April 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, for the purposes of undertaking preclinical and clinical studies in Australia. On April 24, 2019, the Company incorporated Jeune, Inc. in Delaware, a wholly-owned subsidiary, for the purpose of undertaking preclinical studies for aesthetic skin conditions.

We are a clinical stage gene therapy company developing a new class of transformative medicines to treat diseases caused by gene or protein dysfunction or absence. Using our patented platform that is based on engineered herpes simplex virus type 1 ("HSV-1"), we create vectors that encode functional proteins. Our vector is designed to be specifically and efficiently delivered to the target cell in an outpatient setting, via topical, intradermal or inhaled routes of administration, where the cell's own machinery transcribes and translates the encoded protein, restoring or augmenting protein function to treat or prevent disease. We are primarily focused on applying our platform to treat rare monogenic skin conditions caused by insufficient or completely absent protein production. We have expanded our pipeline products to develop medicines to treat chronic, non-monogenic skin diseases and aesthetic skin conditions. Recognizing the breadth and potential transformative power of our HSV-1 vector platform, we have started expanding the scope of our product development beyond skin and have begun preclinical efforts in the field of pulmonary diseases.

Liquidity

As of September 30, 2020, the Company had an accumulated deficit of $60.8 million. With the net proceeds raised from its public and private securities offerings, including the public offering completed in May 21, 2020, the Company believes that its cash, cash equivalents and short-term investments of approximately $286.4 million as of September 30, 2020 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 accounting principles generally accepted 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"). All intercompany balances and transactions have been eliminated in consolidation. Certain prior period amounts have been reclassified to conform to the current period presentation. The reclassified amounts have no impact on the Company's previously reported financial position or results of operation.

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, 2019, as filed with the SEC on March 10, 2020.

Risks and Uncertainties

The pandemic caused by an outbreak of a new strain of coronavirus ("COVID-19") has resulted, and is likely to continue to result, in significant national and global economic disruption and may adversely affect our business. The Company is actively monitoring the impact of COVID-19 and the possible 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 2020.

Use of Estimates

The preparation of financial statements in conformity with U.S. 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, incremental borrowing rate for lease liability, 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 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 US Treasury bills and certificates of deposit. Investments with maturities of greater than one year are classified as long-term investments on the consolidated balance sheets and consist of certificates of deposit. Accrued interest on US Treasury bills and certificates of deposit 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 investments, which consist of US Treasury bills and certificates of deposit, 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	shorter of 8 years or remaining life of lease

Construction-in-progress 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, 2020 and 2019.

Leases

We have entered into lease agreements for our laboratory, manufacturing and office spaces. On January 1, 2019, we adopted ASC 842 – Leases. Pursuant to ASC 842, all of our leases outstanding on January 1, 2019 continued to be classified as operating leases. With the adoption of ASC 842, we recorded an operating lease right-of-use asset of $1.1 million and an operating lease liability of $1.4 million on the condensed consolidated balance sheet. Right-of-use lease assets represent our right to use the 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 were 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. The Company adopted the new lease standard as of the effective date of January 1, 2019, with no restatement of prior periods or cumulative adjustment to retained earnings. Upon adoption, the Company took advantage of the transition package of practical expedients permitted within ASC 842, which allowed the Company not to reassess previous accounting conclusions around whether arrangements were, or contained, leases, as well as to carry forward both the historical classification of leases and the treatment of initial direct costs for existing leases. 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.

Research and Development Expenses

Research and development costs are charged to expense as incurred in performing research and development activities. The 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 and manufacturing organizations that manufacture materials used in the Company's ongoing preclinical and clinical studies. Nonrefundable 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 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 is also required to estimate forfeitures at the time of grant and to revise those estimates in subsequent periods if actual forfeitures differ from its estimates. To the extent that actual forfeitures differ from the Company's estimates, the differences are recorded as a cumulative adjustment in the period the estimates were revised.

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

In August 2018, the FASB issued ASU 2018-13 - Fair Value Measurement (Topic 820) ("ASU 2018-13") which removes, modifies and adds disclosure requirements on fair value measurements. ASU 2018-13 removes disclosure requirements for transfers between Level 1 and Level 2 measurements and valuation processes for Level 3 measurements but adds new disclosure requirements including changes in unrealized gains/losses in other comprehensive income related to recurring Level 3 measurements. The amended guidance was effective for us commencing in the first quarter of 2020. Certain aspects may be applied prospectively while other aspects may be applied retrospectively upon the effective date. The adoption of the guidance resulted in us disclosing the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of September 30, 2020 and 2019.

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. Stock options are common share equivalents. There were 853,336 and 452,311 common share equivalents outstanding as of September 30, 2020 and 2019, respectively, in the form of stock options, 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.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
(In thousands, except shares and per share data)	**(Unaudited)**		**(Unaudited)**	
Numerator:				
Net loss per common share	$ (9,610)	$ (4,272)	$ (21,784)	$ (13,731)
Denominator:				
Weighted-average basic and diluted common shares	19,676,016	17,291,245	18,477,495	15,420,995
Basic and diluted net loss per common share	$ (0.49)	$ (0.25)	$ (1.18)	$ (0.89)

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, 2020 and December 31, 2019, respectively (in thousands):

	September 30, 2020 (unaudited)						
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities [1]	Long-term Marketable Securities [2]
Level 1:							
Cash	$ 3	$ —	$ —	$ 3	$ 3	$ —	$ —
Money market instruments	282,366	—	—	282,366	282,366	—	—
Subtotal	282,369	—	—	282,369	282,369	—	—
Level 2:							
U.S. government agency securities	501	2	—	503	—	503	—
Certificates of deposit	3,474	19	—	3,493	—	3,493	—
Subtotal	3,975	21	—	3,996	—	3,996	—
Total	$ 286,344	$ 21	$ —	$ 286,365	$ 282,369	$ 3,996	$ —

	December 31, 2019						
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities [1]	Long-term Marketable Securities [2]
Level 1:							
Cash	$ 3	$ —	$ —	$ 3	$ 3	$ —	$ —
Money market instruments	187,511	—	—	187,511	187,511	—	—
Subtotal	187,514	—	—	187,514	187,514	—	—
Level 2:							
U.S. government agency securities	1,747	6	—	1,753	—	1,753	—
Certificates of deposit	4,911	4	—	4,915	—	4,418	497
Subtotal	6,658	10	—	6,668	—	6,171	497
Total	$ 194,172	$ 10	$ —	$ 194,182	$ 187,514	$ 6,171	$ 497

(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, 2020	December 31, 2019
	(Unaudited)	
Construction-in-progress	$ 9,389	$ 2,431
Leasehold improvements	4,712	3,179
Furniture and fixtures	854	99
Computer equipment and software	67	45
Laboratory equipment	4,457	3,571
Total property and equipment	19,479	9,325
Accumulated depreciation and amortization	(1,967)	(850)
Property and equipment, net	$ 17,512	$ 8,475

Depreciation expense was $399 thousand and $1.1 million for the three and nine months ended September 30, 2020, respectively, and $190 thousand and $498 thousand for the three and nine months ended September 30, 2019, respectively.

Accrued Expenses and Other Current Liabilities

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

	September 30, 2020	December 31, 2019
	(Unaudited)	
Accrued preclinical and clinical expenses	$ 969	$ 977
Accrued professional fees	358	24
Accrued payroll and benefits	1,096	510
Accrued taxes	30	40
Accrued construction in progress	1,577	263
Other current liabilities	13	12
Total	$ 4,043	$ 1,826

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 was amended to increase the area leased to approximately 31,000 square feet and to extend the expiration date to February 28, 2027, including 6,000 square feet relating to a month-to-month lease that we utilized through February 2020.

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”). The 150,000 square foot facility is under construction and is expected to be completed and validated in 2022. The lease will commence when the space is available for access, which is anticipated to be in 2H 2020, and has an initial term that expires on October 31, 2035. The ASTRA lease contains 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”). The Purchase Option may be

exercised by the Company at any time prior to the date that is thirty days after the initial delivery date, as defined in the lease as the date in which certain delivery conditions have been met by the Landlord. 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, is recorded as prepaid rent in the condensed consolidated balance sheet as of September 30, 2020. Refer to Note 10 for additional information.

As of September 30, 2020, future minimum commitments under the Company's operating leases were as follows (in thousands):

	Operating Leases
2020 (remaining three months)	$ 274
2021	1,358
2022	1,385
2023	1,413
2024	1,441
Thereafter	11,316
Future minimum operating lease payments	$ 17,187
Less: Operating lease payments for ASTRA	13,195
Less: Interest	895
Present value of lease liability	$ 3,097

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

	(unaudited)	
	September 30, 2020	September 30, 2019
Operating leases:		
Right-of-use asset	$ 2,476	$ 2,796
Current portion of lease liability	564	462
Lease liability	2,533	2,860
Total lease liability	$ 3,097	$ 3,322
Weighted average remaining lease term, in years	6.4	7.4
Weighted average discount rate	8.0 %	8.0 %

The Company recorded operating lease costs of $148 thousand and $458 thousand for the three and nine months ended September 30, 2020 and $167 thousand and $449 thousand for the three and nine months ended September 30, 2019, respectively, and variable lease costs of $60 thousand and $88 thousand for the three and nine months ended September 30, 2020 and $9 thousand and $26 thousand for the three and nine months ended September 30, 2019.

Clinical Supply and Product Manufacturing Agreements

The Company has entered into various product manufacturing and clinical supply agreements with Contract Manufacturing Organizations ("CMOs") for the manufacture of clinical trial materials and Contract Research Organizations ("CROs") for clinical trial services. The product manufacturing and clinical supply agreements provide the terms and conditions under which the CMOs and CROs will formulate, fill, inspect, package, label and test our drug product candidates, B-VEC and KB105 for clinical supply. The Company is obligated to make milestone payments. Additionally, certain raw materials, supplies, outsourced testing and other services for the purposes of batch production will be invoiced separately by the CMOs. The estimated remaining commitment as of September 30, 2020 under these agreements for the manufacturing of our drug product is approximately $4.8 million. The Company is also 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 $1.3 million and $2.3 million for the three months and nine months ended September 30, 2020, respectively, and $1.0 million and $3.0 million for the three and nine months ended September 30, 2019, respectively.

Other Contractual Obligations

The Company has contracted with various third parties to facilitate, coordinate and perform agreed upon 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. Business activities being performed under these contracts primarily include market research and other related activities. The estimated remaining commitment as of September 30, 2020 is $1.2 million. The Company has incurred expenses under these activities of $582 thousand and $1.1 million for the three and nine months ended September 30, 2020, respectively, and zero for the three and nine months ended September 30, 2019, respectively.

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., 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, the Messrs. Wechuck and Krisky filed a motion to dismiss the third-party complaint on various grounds, and we have opposed the motion. Discovery in the case has commenced and is expected to continue into the first half of 2021.

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.

7. Capitalization

Sale of Common Stock

On November 1, 2017, the Company entered into a stock purchase agreement ("the Agreement"), with the Epidermolysis Bullosa Medical Research Foundation, a California not-for-profit corporation ("EBMRF"), and EB Research Partnership, Inc., a New York not-for-profit corporation ("EBRP"), and together with EBMRF, the Purchasers, pursuant to which the Company sold to the Purchasers an aggregate of 70,000 shares of the Company's common stock for a purchase price of $11.00 per share, or the Transaction. The Agreement contains redemption features whereby the Company is required to repurchase all or a portion of the shares at a purchase price of $11.00 per share or the closing trading price of the common stock on the redemption request date, whichever is higher, should the Company cease commercially reasonable efforts to work on the research plan pursuant to the Agreement. As the remaining redemption feature is within the control of the Company, the issued common stock has been classified as permanent equity. The Company has continued to perform work relating to the research plan and does not intend to cease commercially reasonable efforts to do so.

On June 27, 2019, the Company completed a public offering of 2,500,000 shares of its common stock to the public at $40.00 per share. Net proceeds to the Company from the offering were $93.8 million after deducting underwriting discounts and commissions of approximately $6.0 million, and other offering expenses payable by the Company of approximately $216 thousand. On July 3, 2019, the underwriters exercised their option to purchase an additional 353,946 shares of common stock at $40.00 per share for additional net proceeds of $13.3 million after deducting underwriting discounts and commissions of approximately $849 thousand. In connection with the public offering, the Company suspended its "at-the-market" equity offering program ("ATM Facility"), that had previously been put in place in March 2019 which had allowed the Company to sell shares of its common stock for up to $50.0 million in gross proceeds. Following the completion of the offering, $16.8 million remains suspended under this 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 granted to employees vest ratably over a four-year period and options granted to directors of the company vest ratably over one year and four-year periods. Stock options have a life of ten years.

The Company granted 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, and 29,500 and 139,000 stock options to employees and directors of the Company during the three and nine months ended September 30, 2019, 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) [1]
Outstanding at December 31, 2019	420,766	$ 17.71	8.4	$ 15,859
Granted	740,250	$ 46.46		
Exercised	(76,935)	$ 9.43		$ 5,423
Cancelled or forfeited	(230,745)	$ 30.55		
Outstanding at September 30, 2020	853,336	$ 39.93	9.1	$ 6,056
Exercisable at September 30, 2020	104,266	$ 11.54	7.2	$ 3,285

(1) Aggregate intrinsic value represents the difference between the closing stock price of our common stock on September 30, 2020 and the exercise price of outstanding in-the-money options.

The weighted-average grant-date fair value per share of options granted to employees during the three months ended September 30, 2020 was $27.44.

There was $19.0 million of unrecognized stock-based compensation expense related to employees' awards that is expected to be recognized over a weighted-average period of 3.1 years as of September 30, 2020.

The Company has recorded aggregate stock-based compensation expense related to the issuance of stock option awards and restricted stock awards in the condensed consolidated statements of operations for the three and nine months ended September 30, 2020 and 2019 as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
	(unaudited)		(unaudited)	
Research and development	$ 344	$ 154	$ 714	$ 396
General and administrative	1,012	124	1,988	520
Total stock-based compensation	$ 1,356	$ 278	$ 2,702	$ 916

Stock Options Granted: The Company recorded stock-based compensation expense of $1.4 million and $2.7 million for the three and nine months ended September 30, 2020, respectively and $278 thousand and $734 thousand for the three and nine months ended September 30, 2019, 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, 2020 and 2019:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Expected stock price volatility	75 %	74 %	75 %	72 %
Expected term of the award (years)	6.20	6.24	6.20	6.08
Risk-free interest rate	0.34 %	1.63 %	0.66 %	2.20 %
Weighted average exercise price	$ 41.89	$ 38.72	$ 46.46	$ 29.73
Forfeiture rate	10.85 %	10.00 %	10.85 %	10.00 %

Restricted Stock Awards: The Company granted 26,213 and 16,213 restricted stock awards ("RSAs"), on June 1, 2018 to our Chief Executive Officer and Chief Operating Officer, respectively. The RSAs vested ratably over a one-year period and had completely vested as of May 31, 2019. No RSAs were outstanding as of September 30, 2020. The fair value of each restricted stock was $10.30 reflecting the closing price of our common stock on the grant date. The Company recorded stock-based compensation expense related to RSAs of zero for the three and nine months ended September 30, 2020 and zero and $182 thousand for the three and nine months ended September 30, 2019, respectively, within general and administrative expenses in the accompanying condensed consolidated statements of operations.

Shares remaining available for grant under the Company's stock incentive plan were 1,698,412, with a sublimit for incentive stock options of 537,068, at September 30, 2020.

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 the full amount on January 6, 2020.

10. Subsequent Events

On October 15, 2020, the Company gave the Landlord notice of its intent to purchase ASTRA for approximately $9.5 million, subject to the parties entering into a commercially reasonable purchase and sale agreement. The Company currently holds approximately $1.5 million on deposit with the Landlord under the existing lease agreement and intends to apply this deposit as a credit against the purchase price at closing. We also expect that the $2.4 million currently recorded as prepaid rent on the balance sheet would be reclassified to property, plant and equipment as part of the book value of the building. As a result of the purchase, the Company will also take 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 subject to a cumulative 10% escalation clause every 5 years through 2071. The financial statement impact related to the transaction cannot be reasonably estimated as of the date of filing due to the uncertainty of market rates and the timing of the close of purchase.

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 elsewhere 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, 2019, as filed with the SEC, on March 10, 2020.

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.

Forward-looking statements appearing in a number of places throughout this Quarterly Report on Form 10-Q include, but are not limited to, statements about the following, among other things:

- the initiation, timing, progress and results of preclinical and clinical trials for B-VEC (previously "KB103"), KB105, KB301 and any other product candidates, including statements regarding 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;
- the impact that the COVID-19 pandemic and measures to prevent its spread may have on our business operations, access to capital, research and development activities, and preclinical and clinical trials for B-VEC, KB105, KB301 and any other 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;
- our estimates regarding the potential market opportunity for B-VEC, KB105, KB301 and any other product candidates;
- our research and development programs for our product candidates;
- our plans and ability to successfully develop and commercialize our product candidates, including B-VEC, KB105, KB301 and our other product candidates;
- our ability to identify and develop new product candidates;
- our ability to identify, recruit and retain key personnel;
- our commercialization, marketing and manufacturing capabilities and strategy;
- the implementation 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;
- our ability to successfully resolve any intellectual property or other claims that may be brought against us;

- any statements regarding compliance with the listing standards of The NASDAQ Capital Market;
- the impact of laws and regulations; and
- any statements regarding economic conditions, including statements related to the economic fallout from the COVID-19 pandemic and the impact on our business, or performance and any statement of assumptions underlying any of the foregoing.

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 prospectus 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 gene therapy company developing a new class of transformative medicines to treat diseases caused by gene or protein dysfunction or absence. Using our patented platform that is based on engineered HSV-1, we create vectors that encode functional proteins. Our vector is designed to be specifically and efficiently delivered to the target cell in an outpatient setting, via topical or intradermal routes of administration, where the cell’s own machinery transcribes and translates the encoded protein, restoring or augmenting protein function to treat or prevent disease.

Presently, we have two product candidates in the clinic to treat rare skin diseases. We announced initiation of a Phase 3 pivotal trial on our most advanced product candidate, B-VEC, to treat dystrophic epidermolysis bullosa (“DEB”) on July 28, 2020. Details of the pivotal study can be found at www.clinicaltrials.gov under NCT identifier NCT04491604. We expect to complete enrollment in this study in early 2021 and anticipate having top-line data from this trial, as well as filing of a Biologics License Application ("BLA") with the U.S. Food and Drug Administration ("FDA"), in 2021. We initiated the Phase 2 portion of our Phase 1/2 study on our second product candidate, KB105, to treat autosomal recessive congenital ichthyosis (“ARCI”) on August 4, 2020 following a successful recent completion of a Phase 1 trial in adults. Details of the Phase 2 study can be found at www.clinicaltrials.gov under NCT identifier NCT04047732. Nothing included on these websites shall be deemed incorporated by reference into this Quarterly Report on Form 10-Q.

We are also applying our HSV-1 platform towards the development of therapies to treat aesthetic skin conditions. We announced initiation of the Phase 1 clinical study on our third product candidate, KB301, to treat wrinkles and acne scars in on August 25, 2020. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT04540900. During the third quarter of 2020, the United States Patent Office ("USPTO") has granted U.S. Patent No. 10,786,438 which covers pharmaceutical compositions comprising HSV vectors encoding one or more cosmetic proteins, as well as methods of their use for improving skin condition, quality, and/or appearance.

Recognizing the breadth and potential transformative power of our HSV-1 vector platform, we have expanded the scope of our product development beyond skin and have begun preclinical efforts in pulmonary diseases. The large payload capacity, robust tropism to epithelial cells (including human airway epithelia), immune-evasive properties, and manufacturing scalability of our HSV-based vector platform gives us an advantage over other viral vector therapies for pulmonary indications. Our preclinical efforts to date have led to the development of a novel candidate, KB407, for the treatment of Cystic Fibrosis (“CF”), which has been shown to successfully transduce human CF patient-derived epithelial cells and deliver functional cystic fibrosis transmembrane conductance regulator (“CFTR”) in vitro in 2D and 3D organotypic systems, and is amendable to non-invasive inhaled administration in vivo, as indicated by successful delivery to the lungs through the use of a clinically relevant nebulizer in small animal models. Successful delivery and distribution throughout the lung was also observed in a non-human primate study. Based on feedback from regulatory agencies, Investigational New Drug (“IND”) enabling safety and efficacy studies,

including an additional safety study in non-human primates, are underway, and IND filing for KB407 is anticipated in 1H 2021. During the third quarter of 2020, we received a Notice of Allowance for our patent application covering methods of using KB407 for the treatment of Cystic fibrosis and other diseases causing progressive lung destruction, which is expected to issue as US Pat. No. 10,829,529 on November 10, 2020. Additional pulmonary diseases are also being evaluated.

We believe that gene therapy companies should control their manufacturing destiny and that having in-house current good manufacturing practices ("cGMP") facilities allow a gene therapy company to maintain better quality control, shorter lead times, lower costs and better command over intellectual property. Last year, we completed the construction of our own commercial scale cGMP-compliant manufacturing facility, ANCORIS, to enhance supply chain control, increase supply capacity for clinical trials and ensure commercial demand is met in the event that B-VEC and our other product candidates receive marketing approval. The clinical material for the pivotal trial has been produced at ANCORIS and we expect to produce initial commercial launch material of B-VEC will be produced at the same facility. Earlier this year, we announced the ground-breaking of our second commercial gene therapy facility in the Pittsburgh, Pennsylvania area. The ASTRA facility is being designed as a state-of-the-art cGMP manufacturing facility that, beyond providing for expansion of Krystal's current production platform, will allow the in-house incorporation of raw material preparation, excipient manufacturing, testing, packaging, labeling and distribution, fully-integrating all components of the supply chain from starting materials to patient experience. We anticipate that the ASTRA facility will initially be used as a commercial back-up facility for B-VEC in the U.S. and supply ex-U.S. markets. Eventually, the ASTRA facility will be expanded to produce investigational and commercial material for our pipeline products. We have recently expanded our facility design to include additional production, quality control labs for testing and release of product, and administrative and training spaces. We expect the 150,000 square foot facility to be completed and validated in 2022.

We have a rapidly expanding portfolio of issued patents in both the United States and foreign jurisdictions and believe that the granting of these patents, which are entirely owned by the Company, protects our core platform and products based thereupon, affording us the freedom to use our patented platform for the development of novel therapeutics for multiple indications. We continue to advance our IP portfolio actively through the filing of new patent applications, divisionals, and continuations relating to our technologies as we deem appropriate. In addition to our patents, we rely on trade secrets and know-how to develop and maintain our competitive position. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, and obtain and maintain ownership of certain technologies, in part, through confidentiality agreements and intellectual property assignment agreements with our employees, consultants and commercial partners. We also seek to preserve the integrity and confidentiality of our data, trade secrets, and know-how, including by implementing measures intended to maintain the physical and electronic security of our research and manufacturing facilities, as well as our information technology systems.

Our desire is to bring transformative medicines, using our platform, to patients suffering from debilitating diseases and conditions. A brief overview of our pipeline follows below.

Pipeline

Beremagene Geperpavec ("B-VEC") for the treatment of Dystrophic Epidermolysis Bullosa

Our lead product candidate, B-VEC, is a topical gene therapy to treat DEB, a rare and severe monogenic skin disease for which there is currently no approved treatment. DEB affects the skin and mucosal tissues and is caused by one or more mutations in a gene called COL7A1, which is responsible for the formation of the protein type VII collagen, or COL7, that forms anchoring fibrils that bind the dermis, or inner layer of the skin, to the epidermis, or outer layer of the skin. In DEB patients, the genetic defect in COL7A1 results in loss or malfunctioning of these anchoring fibrils, leading to extremely fragile skin that blisters and tears from minor friction or trauma. Those who are born with DEB are sometimes called "butterfly children," because their skin is likened to be as fragile as the wings of a butterfly. DEB patients may suffer from open wounds, skin infections, fusion of fingers and toes and gastrointestinal tract problems throughout their lifetime, and may eventually develop squamous cell carcinoma, a potentially fatal condition.

On July 28, 2020, we announced initiation of our Phase 3 pivotal study known as GEM-3. The trial is a randomized, double-blind, intra patient placebo-controlled multicenter study designed to evaluate the efficacy and safety of B-VEC for patients suffering from both recessive and dominant forms of DEB. The trial aims to enroll approximately thirty (30) participants with DEB, aged 6 months or older at time of consent. Investigator identified wound pairs, up to three in each patient, are deemed the "primary" wounds. These primary wounds will be treated once weekly for six months with either B-VEC or placebo, until wound closure. If a wound were to re-open at any point during the study, weekly dosage will resume until closure. The dose administered to each wound is dependent on the size of the wound and ranges from $4x10^8$ to $1.2x10^9$ PFU per wound. A maximum vector dose per patient per week has been defined on the basis of preclinical and clinical safety data. In the event that the maximum dose per patient has not been reached based on dosing of the primary wounds, the study

investigators and patients will have the opportunity to select additional "secondary" wounds across which the remaining weekly dose may be applied.

The Primary Outcome Measure is complete wound healing determined by the Investigator, as compared to baseline in B-VEC treated wounds versus placebo treated wounds at Weeks 20, 22 and 24. Secondary endpoints to be evaluated in the study include complete wound healing at Weeks 8, 10 and 12; the mean change in pain severity (using either a VAS or FLACC-R Scale) per primary wound site associated with wound dressing; the proportion of primary wound sites with ≥75% healing assessed via Canfield photography. Additional exploratory measures include relative time to wound closure from baseline, duration of wound closure, mean reduction in wound surface area in B-VEC treated versus placebo treated wounds, mean change in Quality of Life in addition to Skindex score as compared to baseline at Week 24. Throughout the study, participants will complete questionnaires, have images captured of their study wounds, undergo physical exams, have vital signs and safety labs monitored. Additional details on the study protocol are available at www.clinicaltrials.gov under NCT identifier NCT04491604. Nothing included on this website shall be deemed incorporated by reference into this Quarterly Report on Form 10-Q. We expect to complete enrollment in this study in early 2021 and anticipate having top-line data from this trial as well as filing of a BLA with the FDA in 2021. We are aligned with the European Medicines Agency ("EMA") on a pivotal trial design and we believe that data from GEM-3 will form the basis of a Marketing Authorisation Application ("MAA") filing, shortly after the BLA.

In May of 2020, complete Phase 1/2 data from the GEM-1 and GEM-2 studies was presented at the Society of Investigational Dermatology ("SID") meeting. The Phase 1 portion of the trial commenced in May 2018 at Stanford University, and we announced positive interim results from this clinical study on two patients in October 2018. The Phase 2 portion of the trial commenced in December 2018 at Stanford University, and we announced positive interim results from this clinical study on June 24, 2019.

The FDA and the EMA have each granted B-VEC orphan drug designation for the treatment of DEB, and the FDA has granted B-VEC fast track designation and rare pediatric designation for the treatment of DEB. In addition, in 2019, the FDA granted Regenerative Medicine Advanced Therapy ("RMAT") to B-VEC for the treatment of DEB and the EMA granted PRIority MEdicines, or PRIME, eligibility for B-VEC to treat DEB. The PRIME designation is awarded by the EMA to promising medicines that target an unmet medical need.

KB105 for the treatment of Autosomal Recessive Congenital Ichthyosis

Our second pipeline candidate, KB105, delivers functional human transglutaminase 1 ("TGM1"), genes using our gene therapy platform to patients with TGM1-deficient ARCI. ARCI is a life-long, severe monogenic skin disease. While a number of genetic mutations have been associated with the development of ARCI, the most common cause of ARCI is an inactivating mutation in the TGM1 gene encoding the enzyme transglutaminase-1, a protein that is essential for the proper formation of the skin barrier. Mutations in the TGM1 gene, and the subsequent disruption to the epidermal barrier, leads to pronounced dehydration and trans-epidermal exposure to unwanted toxins and surface microorganisms, greatly increasing the risk of infection and sepsis. Transglutaminase-1 deficiency is associated with increased mortality in the neonatal period and has a dramatic impact on quality of life. There are currently no treatments targeting molecular correction of this disease.

In August 2020, we initiated the second phase of our Phase 1/2 clinical trial of KB105 to treat ARCI. We have enrolled one patient in whom four rectangular 100cm2 (4-inch x 4-inch) areas of skin were selected as Target Areas. Two sites will receive an initial and a repeat dose of 4.0 x 10^9 PFU/Treated Area (TA) while the other two sites will receive 1.0 x 10^10 PFU/ TA. The primary objective of the study is to assess the improvement in localized severity of disease through an Investigator's Global Assessment ("IGA") of disease severity in the treatment area and TGM1 expression and activity and to evaluate safety through the incidence of adverse events associated with KB105 post administration. Additional details on the study protocol are available at www.clinicaltrials.gov under NCT identifier NCT04047732. Nothing on this website shall be deemed incorporated into this Quarterly Report on Form 10-Q.

In May 2020, clinical data from the first phase of the Phase 1/2 study which enrolled adult patients were presented at the SID meeting. Additional details on the interim results are available at http://ir.krystalbio.com/index.php/news-releases/news-release-details/krystal-biotech-announces-positive-interim-results-phase-12. We announced that the study was initiated on September 4, 2019. Nothing on this website shall be deemed incorporated into this Quarterly Report on Form 10-Q.

KB301 for the treatment of aesthetic skin conditions

The skin is largely composed of collagen-rich connective tissue, with dermal collagen, composed primarily of types 1 and 3 collagen fibrils, representing >90% (dry weight) of human skin. The characteristics of skin aging are largely due to aberrant collagen homeostasis, including reduced collagen biosynthesis, increased collagen fibril fragmentation, and progressive loss of dermal collagen culminating in a net collagen deficiency, resulting from both intrinsic (e.g., passage of time,

genetics) and extrinsic (e.g., chronic light exposure, pollution) pressures. The goal of skin biorejuvenation is, in part, to enhance the synthesis of human dermal collagens (i.e., neocollagenesis), thereby correcting the molecular defect underlying the aged phenotype. We believe that our approach of directed expression of full-length human type 3 collagen via intradermal application of KB301 provides a unique and straightforward approach to restoring collagen homeostasis, and by extension, reconstructing an optimal physiologic environment in the skin to treat wrinkles and other superficial skin defects.

We initiated the Phase 1 safety clinical trial for the treatment of wrinkles and acne scars on August 25, 2020. On October 8, 2020, we announced presentation of preclincial data supporting the ongoing development of KB301 at the American Society for Dermatologic Surgery ("ASDS") 2020 Virtual Meeting.

KB104 for the treatment of Netherton Syndrome

KB104 is designed to deliver functional Serine Protease Inhibitor Kazal-type 5 (“SPINK5”), genes using our gene therapy platform to patients suffering from Netherton Syndrome, which is a debilitating monogenic autosomal recessive skin disorder that causes defective keratinization, severe skin barrier defects, and recurrent infections. Severe Netherton Syndrome symptoms in infants are associated with failure to thrive, hypernatremic dehydration secondary to excess fluid loss, delayed growth, short stature, and recurrent infections. Clinically, Netherton Syndrome is characterized by congenital ichthyosiform erythroderma, hair shaft defects, recurrent infections, and a defective skin barrier. A predisposition to allergies, asthma, and eczema is also characteristic of Netherton Syndrome. Ultimately, those afflicted by Netherton Syndrome often experience chronic skin inflammation, severe dehydration, and stunted growth.

KB407 for the treatment of Cystic Fibrosis

We are developing KB407 as a non-invasive inhaled gene therapy product for the treatment of CF and are currently in the preclinical phase with plans for a clinical study for KB407 in 1H 2021. The FDA granted Orphan Drug Designation to KB407 on August 17, 2020, and Rare Pediatric Designation on September 28, 2020.

CF, the most common inherited genetic disorder in the United States, is caused by mutations in the gene encoding CFTR. Lack of functional CFTR in secretory airway epithelia results in defective Cl-, bicarbonate, and thiocyanate secretion, coupled with enhanced Na+ absorption and mucus production, leading to dehydration and acidification of the airway surface liquid. CF is characterized by recurrent chest infections, increased airway secretions, and eventually, respiratory failure. While CF comprises a multiorgan pathology affecting the upper and lower airways, gastrointestinal and reproductive tracts, and the endocrine system, the primary cause of morbidity and mortality in CF is due to progressive lung destruction. According to the US Cystic Fibrosis Foundation (“CFF”), the median age at death for patients with CF in the United States was 30.8 years in 2018. Currently approved CFTR modulating therapies are limited to patients with specific genetic mutations and there is a significant unmet medical need for patients with CF who have genetic mutations non-amenable to currently approved CFTR small molecule “modulators”. According to the CFF, approximately 30,000 patients in the United States and more than 70,000 patients worldwide are living with CF, and approximately 850 new cases of CF were diagnosed in 2018.

Other

In December 2019, COVID-19 was first reported in Wuhan, China and in March 2020, a global pandemic was declared by the World Health Organization. In an effort to slow the spread of the virus, certain governments, including 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 in some areas, 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 COVID-19’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 COVID-19 on our business and clinical trials has been minimal, we will continue to assess the potential impact of the COVID-19 pandemic on our business and operations, including our supply chain and preclinical and clinical trial activities.

At September 30, 2020, our cash, cash equivalents and short-term investments balance was approximately $286.4 million. Since operations began, we have incurred operating losses. Our net losses were $9.6 million and $21.8 million for the three and nine months ended September 30, 2020 and $4.3 million and $13.7 million for the three and nine months ended September 30, 2019, respectively. At September 30, 2020, we had an accumulated deficit of $60.8 million. We will need to generate significant revenue to achieve profitability, and we may never generate revenue or enough revenue to achieve profitability. We expect to incur significant expenses and increasing operating losses for the foreseeable future. Our net losses

may fluctuate significantly from quarter to quarter and year to year. We expect our costs will continue to increase significantly as a result of our current and planned business activities, such as:

- conducting our Phase 3 clinical trial for B-VEC, Phase 1/2 clinical trial for KB105, and Phase 1 clinical trial for KB301;
- continued research and development-related activities for the advancement of our pipeline product candidates into clinical development, such as KB104 and KB407;
- construction of our cGMP manufacturing facility, ASTRA, and related completion and validation costs;
- manufacturing of our clinical trial materials;
- pursuing regulatory approval for our product candidates;
- adding personnel to support our administrative, product development and commercialization efforts; and
- activities leading up to the commercial launch of B-VEC in multiple markets.

Costs related to clinical trials can be unpredictable and therefore there can be no guarantee that we will have sufficient capital to fund our planned preclinical studies for our pipeline product candidates, or our operations. Our funds may not be sufficient to enable us to seek marketing approval for or to commercially launch B-VEC, KB105, KB301 or any other product candidate. Accordingly, to obtain marketing approval for and to commercialize this 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 planned business strategy.

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 begin our pivotal Phase 3 clinical trial for B-VEC, conduct our ongoing Phase 1/2 clinical trial for KB105, 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.

Interest Income

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

Critical Accounting Policies, Significant Judgments and Estimates

There have been no significant changes during the three and nine months ended September 30, 2020 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, 2019.

Results of Operations

Three Months Ended September 30, 2020 and 2019

	Three Months Ended September 30,		
	2020	2019	Change
(In thousands)	**(unaudited)**		
Expenses			
Research and development	$ 5,100	$ 3,885	$ 1,215
General and administrative	4,580	1,457	3,123
Total operating expenses	9,680	5,342	4,338
Loss from operations	(9,680)	(5,342)	(4,338)
Other Income			
Interest and other income, net	70	1,070	(1,000)
Net loss	$ (9,610)	$ (4,272)	$ (5,338)

Research and Development Expenses

Research and development expenses increased $1.2 million in the three months ended September 30, 2020 compared to the three months ended September 30, 2019. Higher research and development expenses were due to an increase in outsourcing research and development activities of $352 thousand, lab supplies of $187 thousand, payroll related expenses of $526 thousand which is primarily driven by an increase in headcount to support overall growth and includes a $190 thousand increase in stock-based compensation, and other research and development expenses of $150 thousand.

General and Administrative Expenses

General and administrative expenses increased $3.1 million in the three months ended September 30, 2020 as compared to the three months ended September 30, 2019. Higher general and administrative spending was due largely to increases in payroll related expenses of approximately $1.6 million which is primarily driven by an increase in headcount to support overall growth and includes an $888 thousand increase in stock-based compensation, market research related expenses of $611 thousand, legal and professional fees of $631 thousand and other administrative expenses of $298 thousand.

Interest and Other Income

Interest and other income for the three months ended September 30, 2020 and 2019 was $70 thousand and $1.1 million, respectively and consisted of interest and dividend income earned from our cash, cash equivalents and investments. This decrease was driven by a decline in market interest rates.

Nine Months Ended September 30, 2020 and 2019

	Nine Months Ended September 30,		
	2020	2019	Change
(In thousands)	**(unaudited)**		
Expenses			
Research and development	$ 12,264	$ 11,267	$ 997
General and administrative	10,315	4,660	5,655
Total operating expenses	22,579	15,927	6,652
Loss from operations	(22,579)	(15,927)	(6,652)
Other Income			
Interest and other income, net	795	2,196	(1,401)
Net loss	$ (21,784)	$ (13,731)	$ (8,053)

Research and Development Expenses

Research and development expenses increased $997 thousand in the nine months ended September 30, 2020 compared to the nine months ended September 30, 2019. Higher research and development expenses were due largely to an increase in payroll related expenses of approximately $1.4 million which is primarily driven by an increase in headcount to support overall growth and includes a $318 thousand increase in stock-based compensation and a net increase in other research and development expense of $311 thousand offset by an decrease in outsourcing research and development activities of $681 thousand.

General and Administrative Expenses

General and administrative expenses increased $5.7 million in the nine months ended September 30, 2020 as compared to the nine months ended September 30, 2019. Higher general and administrative spending was due largely to increases in payroll related expenses of approximately $3.1 million which is primarily driven by an increase in headcount to support overall growth and includes an approximately $1.5 million increase in stock-based compensation, market research related expenses of approximately $1.1 million, legal and professional fees of $748 thousand, insurance expense of $552 thousand and other administrative expenses of $177 thousand.

Interest and Other Income

Interest and other income for the nine months ended September 30, 2020 and 2019 was $795 thousand and $2.2 million, respectively and consisted of interest and dividend income earned from our cash, cash equivalents and investments. This decrease was driven by a decline in market interest rates.

Liquidity and Capital Resources

Overview

As of September 30, 2020, the Company had an accumulated deficit of $60.8 million. With the net proceeds raised from its public and private securities offerings, including the public offering completed on May 21, 2020, the Company believes that its cash, cash equivalents and short-term investments of approximately $286.4 million as of September 30, 2020 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 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. 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. There can be no assurances that additional funding will be available on terms acceptable to the Company, if at all. In addition, the COVID-19 pandemic may negatively impact our operations, including possible effects on its financial condition, ability to access the capital markets on attractive terms or at all, liquidity, operations, suppliers, industry, and workforce. 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 2020 and beyond.

Operating Capital Requirements

We expect our primary use of capital to continue to be for 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. We believe that our available funds will be sufficient to enable us to complete our pivotal Phase 3 clinical trials for B-VEC, to continue our Phase 1/2 clinical trials for KB105, to complete our Phase 1 clinical trials for KB301, as well as to continue construction and validation related activities associated with our cGMP manufacturing facility, ASTRA.

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 facility;
- 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 B-VEC, KB105, KB301 or any other product candidates we may develop;
- 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 B-VEC, KB105, KB301 and other 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 B-VEC, KB105, KB301 or our other 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 may need to obtain substantial additional funding in order to receive regulatory approval and to commercialize B-VEC or any other product candidates, including KB105. 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 B-VEC, KB105, KB301 or our other 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 B-VEC, KB105, KB301 or our other 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):

	Nine months Ended September 30,	
	2020	**2019**
	(unaudited)	
Net cash used in operating activities	$ (18,059)	$ (11,690)
Net cash used in investing activities	(4,964)	(4,400)
Net cash provided by financing activities	117,878	107,226
Net increase in cash	$ 94,855	$ 91,136

Operating Activities

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 primarily of depreciation and amortization and stock-based compensation expense of $4.1 million, and cash used by increases in net operating assets of approximately $371 thousand.

Net cash used in operating activities for the nine months ended September 30, 2019 was $11.7 million and consisted primarily of a net loss of $13.7 million adjusted for non-cash items of depreciation and amortization and stock-based compensation expense of approximately $1.7 million, and cash provided by decreases in net operating liabilities of $318 thousand.

Investing Activities

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.

Net cash used in investing activities for the nine months ended September 30, 2019 was $4.4 million and consisted primarily of purchases of $6.9 million of short-term available-for-sale investment securities, and expenditures of $4.1 million on the build-out of our new cGMP facilities and purchases of computer and laboratory equipment, partially offset by proceeds of $6.6 million received from the maturities of short-term investments.

Financing Activities

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 on May 21, 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.

Net cash provided by financing activities for the nine months ended September 30, 2019 was $107.2 million and was primarily from proceeds from our public offering in June 2019 of 2,500,000 shares of our common stock at a price to the public of $40.00 per share. Net proceeds to the Company from the offering were $93.8 million after deducting underwriting discounts and commissions of approximately $6.0 million and other offering expenses of approximately $190 thousand. On July 3, 2019, the underwriters exercised their option to purchase an additional 353,946 shares of common stock at $40.00 per share for additional net proceeds of $13.3 million after deducting underwriting discounts and commissions of approximately $849 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, 2019 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, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

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 $286.4 million at September 30, 2020, which consist primarily of money market, bank deposits, U.S. Treasury bills and certificates of deposit. 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 likely decline. To minimize this risk, we intend to maintain a portfolio which may include cash, cash equivalents and 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 have 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-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

There has been no change in our internal control over financial reporting during the quarter ended September 30, 2020 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. We have not experienced any material impact to our internal controls over financial reporting despite the fact that some of our employees are working remotely due to the COVID-19 pandemic. We are continually monitoring and assessing the impact of COVID-19 on our internal controls to minimize the impact on their design and operating effectiveness.

PART II. OTHER INFORMATION

ITEM 1. 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., 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, the Messrs. Wechuck and Krisky filed a motion to dismiss the third-party complaint on various grounds, and we have opposed the motion. Discovery in the case has commenced and is expected to continue into the first half of 2021.

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.

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, 2020, we had an accumulated deficit of $60.8 million. Our ability to achieve profitability depends on our ability to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, B-VEC, KB105, and KB301 and additional product candidates that we may pursue in the future. 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, B-VEC, KB105, and KB301 as well as to building out our infrastructure. We expect that it could be several years, 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 additional clinical trials and preclinical studies for any additional product candidates that we may pursue in the future;
- prepare our BLA, MAA, and approvals in certain other countries for B-VEC, KB105, and KB301;
- ramp-up our in-house commercial-scale cGMP manufacturing facility;
- 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, KB105, KB301 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 B-VEC, KB105, and KB301, developing and validating commercial scale manufacturing processes, obtaining marketing approval for this product candidate, manufacturing, marketing and selling any future product candidates for which we may obtain marketing approval and satisfying any post-marketing requirements. If we were required to discontinue development of either B-VEC, KB105, or KB301, if B-VEC, KB105, or KB301 do not receive regulatory approval, if we do not obtain our targeted indications for B-VEC, KB105, or KB301, or if B-VEC, KB105, or KB301 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 would materially adversely affect our business prospects and financial condition. Moreover, if we decide to leverage any success with our B-VEC, KB105, or KB301 product candidates to develop other product opportunities, we may not be successful in such efforts. In any such event, our business will be materially adversely affected.

We currently only 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 you to lose all or part of your 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 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 will need to raise additional funding in order to receive approval for B-VEC, KB105, KB301 or any other product candidate. 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 B-VEC, KB105, and KB301 and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize B-VEC, KB105, and KB301, if approved, we will require substantial additional funding. In addition, if we obtain marketing approval for B-VEC, KB105, or KB301, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, we expect to continue to incur significant costs associated with operating as a public company. We anticipate that we will 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 on an IND application for 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;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, in the event we receive marketing approval for B-VEC, KB105, KB301 or any other product candidates we may develop;
- 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 B-VEC, KB105, KB301 and other 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 B-VEC, KB105, KB301 or any of our other 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;
- 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.

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 B-VEC, KB105, and KB301 have been limited to organizing and staffing our company, business planning, raising capital, developing our STAR-D platform and related technologies, identifying B-VEC, KB105 and KB301 as potential gene therapy product candidates and undertaking preclinical studies and clinical trials of B-VEC, KB105, and KB301. While we have conducted clinical trials of B-VEC, KB105, and KB301, we have not yet demonstrated the ability to complete clinical trials of B-VEC, KB105, KB301 or any other product candidate, 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 at some point 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 of our product candidates and adversely impact our business.

Public health crises such as pandemics or similar outbreaks could adversely impact our business. In December 2019, a new strain of coronavirus surfaced in Wuhan, China and has reached multiple other regions and countries, including Pittsburgh, Pennsylvania where our primary office, manufacturing and laboratory facilities are located. 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.

Further, in response to the pandemic and in accordance with direction from national, state and local government authorities, we have restricted access to our office, manufacturing and laboratory facilities to personnel and third parties who must perform critical activities that must be completed on-site, limited the number of such personnel that can be present at our facilities at any one time, and requested that many of our personnel work remotely. In the event that governmental authorities were to further modify current 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 or quarantines 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 worsen in countries that are already afflicted with the coronavirus or could continue to spread to additional countries, each of which could further 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 continue to deteriorate as a result of the pandemic such that our access to capital and other sources of funding may be constrained.

The COVID-19 outbreak continues to evolve rapidly. 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 ultimate geographic spread of the disease, 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 B-VEC, KB105, and KB301 through clinical trials, obtain regulatory approval and ultimately commercialize B-VEC, KB105, or KB301, 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 B-VEC, KB105, or KB301 (or any other product candidate we may develop) is 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 B-VEC, KB105, and KB301, if and when approved, whether alone or in collaboration with others;
- acceptance of B-VEC, KB105, and KB301, 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 B-VEC, KB105, and KB301, which would materially harm our business. If we do not receive regulatory approvals for B-VEC, KB105, and KB301, our business, financial condition, results of operations and prospects could be materially and adversely affected.

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. 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 interim 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.

The regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends

non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or post-approval safety monitoring program. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of 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.

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 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. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of 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 European Union or how long it will take to commercialize our product candidates. Approvals by the European Commission may not be indicative of what FDA may require for approval.

Regulatory requirements 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 (“IBC”) 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 will be required to consult with these regulatory and advisory groups and 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. 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, our clinical trials could be suspended or terminated. If in the future 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 of such product candidate may be harmed and our ability to generate product revenues from this product candidate 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 such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- 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 B-VEC, 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 B-VEC 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 B-VEC 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 or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the drug or impose restrictions on its distribution;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- 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 ("GCP") 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 facility 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 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 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. 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 and we may seek orphan drug designation from the FDA for our future product candidates. On April 16, 2018, the European Commission granted the Orphan Medicinal Product Designation ("OMPD") 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 ARCI, and on October 10, 2019, the European Commission granted the Orphan Medicinal Product Designation for KB105. There are currently no treatments for ARCI, which affects approximately 20,000 patients worldwide. On August 17, 2020, the FDA granted orphan drug designation to our most recent product candidate, KB407, currently in pre-clinical 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 candidate 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 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, and KB301, 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 may 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.

We are aware of several companies and institutions that are currently developing alternative autologous or palliative gene therapy approaches for DEB. 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 uneconomical or obsolete, and we may not be successful in marketing B-VEC against competitors.

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

Risks Related to Manufacturing

Delays in obtaining regulatory approvals of the process and facilities needed to manufacture B-VEC, KB105, KB301 or any of 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 B-VEC, KB105, KB301 or any of our product candidates, whether in a third-party facility or in our own facility, once established, we must pass a pre-approval inspection of our manufacturing facility by the FDA before B-VEC, KB105, KB301 or any of 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. In order 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 US 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 B-VEC is 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 B-VEC 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 other product candidates or the clinical trials required for approval of B-VEC. In such instances, we may need to locate an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense prior to the approval of B-VEC 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 B-VEC 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 B-VEC 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 B-VEC, KB105 and KB301, if approved, we plan to expand our capabilities to promote market access and build awareness. To successfully commercialize any other 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.

Our success also will depend upon physicians who specialize in the treatment of DEB prescribing treatments that involve the use of B-VEC, KB105 and KB301, respectively, in lieu of, or in addition to, other treatments with which they are more 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 B-VEC or demand for any product candidate we may develop. 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 B-VEC, stricter labeling requirements for B-VEC if approved and a decrease in demand for B-VEC.

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

We are currently focusing our research and product development efforts 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 B-VEC, KB105, KB301 and any future 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 B-VEC, KB105 and KB301. 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 B-VEC, KB105 and KB301 will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and B-VEC, KB105 and KB301 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 B-VEC, KB105 and KB301, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of B-VEC, KB105 and KB301 as demonstrated in clinical trials;
- the efficacy, potential and perceived advantages of B-VEC, KB105 or KB301 over alternative treatments, if available;
- the cost of B-VEC, KB105 or KB301 relative to alternative treatments, if any are available;
- the clinical indications for which B-VEC, KB105 and KB301 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 B-VEC, KB105 or KB301, if approved, or any of our other product candidates that may be approved in the future, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement of pharmaceutical may be increasingly restricted both in the US 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 US 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 US Specifically, there have been several recent US 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 future 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 these treatments. 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 counties 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 (“ASP”), average manufacturer price (“AMP”), 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 or our partners 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 legislative 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 products candidate, 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. This could lead to governmental authorities prohibiting genetic testing or calling for limits on or regulating the use 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 B-VEC, KB105, KB301 or our future product candidates will be harmed and our business, financial condition, results of operations and prospects will be adversely affected.

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 research may be unsuccessful in identifying potential product candidates or 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 and scientific advisors 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 and scientific advisors are at-will employees and consultants, 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 and scientific advisors 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 and insider trading.

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.

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 US 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 judicial and Congressional challenges to certain aspects of the PPACA. As a result, there have been delays in the implementation of, and action taken to repeal or replace, certain aspects of the PPACA. In January 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the PPACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act ("ACA") that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. Further, on December 14, 2018, US Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12 billion in ACA risk corridor payments to third-party payors who argued were owed to them. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, each chamber of Congress has put forth multiple bills this year designed to repeal or repeal and replace portions of the ACA. While Congress has not passed repeal legislation, the Tax Reform Act includes a provision repealing,

effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Further, the Bipartisan Budget Act of 2018 ("BBA"), among other things, amends the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." Congress may consider other legislation to repeal and replace elements of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Additionally, in the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biologic products that are demonstrated to be "highly similar" or "biosimilar or interchangeable" with an FDA-approved biologic product. This new pathway could allow competitors to reference data from biologic products already approved after 12 years from the time of approval. This could expose us to potential competition by lower cost biosimilars even if we commercialize a product candidate faster than our competitors. Moreover, the creation of this abbreviated approval pathway does not preclude or delay a third party from pursuing approval of a competitive product candidate via the traditional approval pathway based on their own clinical trial data. 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. Additionally, there have been several recent US Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs.

Further, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which have resulted in several recent Congressional inquiries and proposed and enacted bills designed to, among other things, 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.

We expect that 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 B-VEC, KB105, KB301 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 B-VEC, KB105, and KB301 and begin commercializing it 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.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations.

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 for our services 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 B-VEC and KB105 is located at our manufacturing facility in Pittsburgh, Pennsylvania. We were are in the early stages of 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 US and foreign patent protection for our product candidates, including B-VEC, KB105 and KB301, any future product candidates we may develop, and/or our STAR-D 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 and additional product candidates in our pipeline, current and future innovations related to our STAR-D 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 four issued patents in the United States: (1) US 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) US patent No. 10,155,016 covering pharmaceutical compositions containing B-VEC formulated for myriad routes of administration; (3) US patent No. 10,441,614 covering aspects of our STAR-D platform technology, and its uses in delivering any gene of interest to the skin; (4) US 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) US Patent No. 10,786,438 covering pharmaceutical compositions comprising our third product candidate, KB301, and methods for its use for improving skin condition, quality, and/or appearance; and (6) US 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 seven international patent applications filed in accordance with the Paris Cooperation treaty directed to multiple discovery, preclinical, and clinical programs, including both B-VEC, KB105 and KB301, as well as multiple patent applications filed in foreign jurisdictions stemming from these international applications. B-VEC is also the subject of patents granted in both Australia and Europe, 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 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 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 STAR-D 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 will 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. We may in the future become party to, or be threatened with, adversarial proceedings or litigation

regarding intellectual property rights with respect to B-VEC, KB105, KB301 or related technologies, including, for example, interference proceedings, post grant review challenges, and *inter partes* review before the 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 US 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 US patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such US patent. In such a hypothetical situation, there is no assurance that a court of competent jurisdiction would find that B-VEC, KB105, KB301 or our other 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 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 above under “Item 3—Legal Proceedings,” 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 product candidates, including the vector backbones, and our STAR-D platform. 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 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 US 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 US 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 US Court of Appeals for the Federal Circuit and the US 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 currently have not registered 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 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, 2020, 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 19% 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 patent 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 are an "emerging growth company" and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and we may take advantage of certain exemptions and relief from various reporting requirements that are applicable to other public companies that are not "emerging growth companies." In particular, while we are an "emerging growth company: (i) we will not be required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act; (ii) we will be exempt from any rules that may be adopted by the Public Company Accounting Oversight Board requiring mandatory audit firm rotations or a supplement to the auditor's report on financial statements; (iii) we will be subject to reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements; and (iv) we will not be required to hold nonbinding advisory votes on executive compensation or stockholder approval of any golden parachute payments not previously approved. Investors may find our common stock less attractive if we rely on the exemptions and relief granted by the JOBS Act. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may decline or become more volatile.

In addition, the JOBS Act provides that an emerging growth company may take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

We will continue to incur costs as a result of becoming a public company, and such costs may increase if and 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, although we are currently unable to estimate these costs with any degree of certainty. We could be an emerging growth company until the end of 2022, 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. After we are no longer an emerging growth company under the JOBS Act, beginning no later than our year ending December 31, 2023, 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 you might otherwise receive a premium for your 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

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.

Any of these developments could have a material adverse effect on our business, financial condition, and results of operations.

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	Certification of Periodic Report by Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Periodic Report by Chief Accounting Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	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.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

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 6, 2020	By:	/s/ Krish S. Krishnan
		Krish S. Krishnan President and Chief Executive Officer
	By:	/s/ Kathryn A. Romano
		Kathryn A. Romano Chief Accounting Officer

Exhibit 31.1

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 6, 2020

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

Exhibit 31.2

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 6, 2020

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

Exhibit 32.1

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, 2020, (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 6, 2020

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, 2020, (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 6, 2020

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