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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, 2017

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	**82-2908297**
(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)

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

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

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

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☒

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

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 ☒

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date.

There were 10,237,247 shares of the registrant's common stock issued and outstanding as of October 31, 2017.

Krystal Biotech, Inc. (formerly Krystal Biotech, LLC)
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Krystal Biotech, Inc. (formerly Krystal Biotech, LLC)
Condensed Balance Sheets

(In thousands, except shares, units, per share and per unit data)	September 30, 2017 (unaudited)	December 31, 2016
Assets		
Current assets		
Cash	$ 52,570	$ 1,923
Prepaid research and development expenses	48	246
Total current assets	52,618	2,169
Property and equipment, net	55	13
Total assets	$ 52,673	$ 2,182
Liabilities, Convertible Preferred Stock and Stockholders' and Members' Equity (Deficit)		
Current liabilities		
Accounts payable	$ 550	$ 42
Accrued expenses and other current liabilities	1,925	1
Total current liabilities	2,475	43
Accrued interest	—	7
Related party convertible promissory notes	—	698
Convertible promissory notes	—	1,145
Total liabilities	2,475	1,893
Commitments and contingencies (Note 7)		
Convertible preferred stock		
Convertible preferred stock; $0.00001 par value; 20,000,000 shares authorized, 2,061,773 shares issued, and no shares outstanding at September 30, 2017 (unaudited); no shares authorized, issued, or outstanding at December 31, 2016	—	—
Total convertible preferred stock	—	—
Stockholders' and members' equity (deficit)		
Common stock; $0.00001 par value; 80,000,000 shares authorized, 10,237,247 shares issued and outstanding at September 30, 2017 (unaudited); no shares authorized, issued, or outstanding at December 31, 2016	—	—
Common units; no common units authorized, issued, or outstanding at September 30, 2017 (unaudited); no par value; 3,490,884 units authorized, issued, and outstanding at December 31, 2016	—	—
Preferred units; no par value; no preferred units authorized, issued, or outstanding at September 30, 2017 (unaudited); no par value; 179,613 units authorized, issued, and outstanding at December 31, 2016 (aggregate liquidation preference of $1,406)	—	1,406
Additional paid-in capital	57,875	33
Accumulated deficit	(7,677)	(1,150)
Total stockholders' and members' equity	50,198	289
Total liabilities, convertible preferred stock and stockholders' and members' equity	$ 52,673	$ 2,182

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

Krystal Biotech, Inc. (formerly Krystal Biotech, LLC)
Condensed Statements of Operations
(Unaudited)

(In thousands, except shares, units, per share and per unit data)	Three Months Ended September 30, 2017	Three Months Ended September 30, 2016	Nine Months Ended September 30, 2017	Nine Months Ended September 30, 2016
Expenses				
Research and development	$ 1,355	$ 241	$ 2,120	$ 339
General and administrative	738	81	1,154	184
Total operating expenses	2,093	322	3,274	523
Loss from operations	(2,093)	(322)	(3,274)	(523)
Other Expense				
Interest expense, net	(3,180)	—	(3,253)	—
Total other expense	(3,180)	—	(3,253)	—
Net loss	(5,273)	(322)	(6,527)	(523)
Net loss applicable to stockholders and members	$ (5,273)	$ (322)	$ (6,527)	$ (523)
Net loss attributable to common stockholders per share:				
Basic and diluted	$ (1.26)	$ (8.48)	$ (1.75)	$ (40.20)
Weighted-average common shares and common units outstanding				
Basic and diluted	4,183,075	37,982	3,725,825	13,016

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

Krystal Biotech, Inc. (formerly Krystal Biotech, LLC)
Condensed Statements of Cash Flows
(Unaudited)

(In thousands)	Nine Months Ended September 30, 2017	2016
Operating Activities		
Net loss	$ (6,527)	$ (523)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation	9	1
Stock-based compensation expense	188	7
Non-cash interest expense	3,263	—
(Increase) decrease in		
Prepaid research and development expenses	198	(12)
Increase (decrease) in		
Accounts payable	271	62
Accrued expenses and other current liabilities	835	15
Net cash used in operating activities	(1,763)	(450)
Investing Activities		
Purchases of property and equipment	(51)	(6)
Net cash used in investing activities	(51)	(6)
Financing Activities		
Proceeds from the issuance of convertible promissory notes	2,299	—
Issuance of common stock and common units, net	43,162	100
Issuance of preferred stock and preferred units	7,000	754
Net cash provided by financing activities	52,461	854
Net increase in cash	50,647	398
Cash at beginning of period	1,923	—
Cash at end of period	$ 52,570	$ 398
Supplemental Disclosures of Non-Cash Investing and Financing Activities		
Conversion of common units to preferred units	$ —	$ 100
Conversion of convertible promissory notes to preferred stock	$ 4,142	$ —
Conversion of preferred stock to common stock	$ 12,661	$ —
Unpaid deferred offering costs	$ 1,327	$ —

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

Krystal Biotech, Inc. (formerly Krystal Biotech, LLC)
Notes to Unaudited Condensed Financial Statements
(In thousands, except per share data)

1. Organization

Krystal Biotech, Inc. (the "Company," or "we" or other similar pronouns) was formed on December 20, 2015 in the State of California as Krystal Biotech, LLC and began operations on April 15, 2016. On March 31, 2017, the Company converted from a limited liability company ("LLC") to a C-corporation in the state of Delaware, and changed its name to Krystal Biotech, Inc.

Stock Split and Increase in Authorized Shares

On September 5, 2017, in connection with our initial public offering (the "IPO"), the Company's board of directors (the "Board") approved a 1-to-4.5 forward stock split, in the form of a dividend, of all outstanding shares of common stock and preferred stock. Except as otherwise noted, all references to share and per share amounts related to common stock, common units, preferred stock, preferred units and stock options in these condensed financial statements reflect the stock split. The par value per share of our capital stock was not adjusted as a result of the stock split. Additionally, the Board approved an increase in authorized shares of common stock and preferred stock to 80,000,000 shares and 20,000,000 shares, respectively. The stock split and the increase in the number of authorized common and preferred shares occurred immediately prior to the effectiveness of our registration statement on Form S-1 (File No. 333-220085) relating to the IPO on September 19, 2017.

Initial Public Offering

On September 22, 2017, the Company completed its initial public offering of 4,554,000 shares of its common stock at a price to the public of $10.00 per share, which includes the sale of 594,000 shares of the Company's common stock pursuant to the underwriters' full exercise of their option to purchase additional shares. The total proceeds from the offering to the Company, net of underwriting discounts and commissions of approximately $3.2 million, were approximately $42.3 million. After deducting offering expenses payable by the Company of approximately $1.5 million, net proceeds to the Company were approximately $40.8 million. Immediately prior to the closing of the IPO, all outstanding shares of the Company's preferred stock converted into 2,061,773 shares of common stock on a 1-to-1 basis.

Liquidity and Risks

As of September 30, 2017 and December 31, 2016, the Company had accumulated deficits since inception of $7.7 million and $1.2 million, respectively. With the net proceeds raised upon the close of IPO, the Company believes that its cash of approximately $52.6 million as of September 30, 2017 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 or obtain financing from other sources, such as partnerships. 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 assurances, however, that additional funding will be available on terms acceptable to the Company, or at all.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development of technological innovations by its competitors, risks of failure of clinical studies, dependence on key personnel, protection of proprietary technology, compliance with government regulations, and ability to transition from preclinical manufacturing to commercial production of products.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying condensed financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

Unaudited Condensed Financial Information

The accompanying condensed balance sheet as of September 30, 2017, the condensed statements of operations for the three and nine months ended September 30, 2017 and 2016, and condensed statements of cash flows for the nine months ended September 30, 2017 and 2016, and the related information contained within the notes to the condensed financial statements are unaudited. As permitted under GAAP for interim financial information under instructions to Form 10-Q and Article 10 of Regulation S-X., certain footnotes or other financial information can be condensed or omitted. These financial statements and related disclosures have been prepared with the assumption that users of the interim financial information have read or have access to the audited financial statements for the preceding fiscal year. Accordingly, these statements should be read in conjunction with the audited consolidated financial statements and related notes included in our registration statement on Form S-1 relating to the IPO on September 19, 2017. The condensed financial statements have been prepared on the same basis as the annual audited financial statements and, in the opinion of management, reflect all adjustments, consisting of normal and recurring adjustments, necessary for the fair presentation of the Company's financial position at September 30, 2017, the statements of operations for the three and nine months ended September 30, 2017 and 2016, and its cash flows for the nine months ended September 30, 2017 and 2016. The results for the three and nine months ended September 30, 2017 are not necessarily indicative of results to be expected for the year ending December 31, 2017 or any other condensed or future period.

Use of Estimates

The preparation of condensed financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the condensed 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 condensed 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 condensed financial statements. Estimates are used in the following areas, among others: stock-based compensation expense, accrued research and development expenses, the fair value of financial instruments, and the valuation allowance included in the deferred income taxes calculations.

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. The Company operates in only one geographic segment.

Concentrations of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially subject the Company to credit risk consist of cash. The Company's policy is to invest its cash in money market funds and various bank deposit accounts. These financial instruments were held in Company accounts at two financial institutions. 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 of amounts recorded on the balance sheets which may be 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 consists of money market funds and bank deposit.

Deferred Issuance Costs

Deferred public offering costs, which consist primarily of direct, incremental legal and accounting fees relating to the IPO, are capitalized within other assets at December 31, 2016 and are offset against IPO proceeds at September 30, 2017.

Fair Value of Financial Instruments

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. FASB ASC Topic 820, *Fair Value Measurement and Disclosures*, established a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the financial instrument based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the financial instrument

and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported or disclosed fair value of the financial instruments and is not a measure of the investment credit quality. Fair value measurements are classified and disclosed in one of the following three categories:

- *Level 1*—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- *Level 2*—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- *Level 3*—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that 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 in 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 changes to the valuation methods utilized by the Company during the nine months ended September 30, 2017 and the year ended December 31, 2016. The Company evaluates transfers between levels at the end of each reporting period. There were no transfers of financial instruments between levels during the nine months ended September 30, 2017 and the year ended December 31, 2016.

The carrying amounts of financial instruments consisting of cash, prepaid expenses, accounts payable and accrued expenses, related party convertible promissory notes and convertible promissory notes included in the Company's Condensed financial statements are reasonable estimates of fair value due to their short maturities.

Property and Equipment, net

Property and equipment 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 years

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. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value. The Company has not recognized any impairment losses through September 30, 2017.

Convertible Preferred Stock

In accordance with the guidance in FASB ASC Topic 480, *Distinguishing Liabilities from Equity*, shares of preferred stock (Note 7), were classified outside of permanent equity and within temporary equity, as of December 30, 2016 due to their associated redemption features and liquidation preferences. There were no shares of preferred stock outstanding as of September 30, 2017 and December 30, 2016.

The Company evaluated its preferred stock and determined that it is considered an equity host under ASC 815. In making this determination, the Company's analysis followed the whole instrument approach which compares an individual feature against the entire preferred stock instrument which includes that feature. The Company's analysis was based on a consideration of the economic characteristics and risks of the preferred stock. More specifically, the Company evaluated all of the stated and implied substantive terms and features, including: (i) whether the preferred stock included redemption features; (ii) how and when any redemption features could be exercised; (iii) whether the holders of the preferred stock were entitled to dividends and how those dividends were calculated; (iv) the voting rights of the Preferred stock; and (v) the existence and nature of any conversion rights. As a result of the Company's conclusion that the preferred stock both represent an equity host, the redemption features of the preferred stock are considered to be clearly and closely related to the associated equity host instruments. Accordingly, the redemption features of the preferred stock are not considered embedded derivatives that require bifurcation. The Company also concluded that the conversion rights under the preferred stock are clearly and closely related to the equity host instruments and are not considered embedded derivatives that require bifurcation.

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 activities and related clinical manufacturing costs, 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 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 to employees and directors in accordance with FASB ASC Topic 718, *Compensation-Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, including grants of employee stock options and restricted stock, to be recognized in the statements of operations based on their grant date fair values. Compensation expense related to awards to employees 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. Share-based payments issued to non-employees are recorded at their fair values, and are periodically revalued as the equity instruments vest and are recognized as expense over the related service period in accordance with the provisions of ASC 718 and ASC Topic 505, *Equity,* and are expensed using an accelerated attribution model. 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; (iv) expected dividends; and (v) the estimated fair value of its common stock on the measurement date. Due to the lack of a public market for the trading of its 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 average of the vesting term and the original contractual term of the option. The expected term for non-employee awards is the remaining contractual term of the option. The risk-free interest rates are based on the U.S. 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 revise those estimates in subsequent periods if actual forfeitures differ from its estimates. The Company uses historical data to estimate forfeitures and records stock-based compensation expense only for those awards that are expected to vest. 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. Stock-based compensation expense recognized in the Condensed financial statements is based on awards that are ultimately expected to vest.

Net Loss Per Share Attributable to Common Stockholders and Members

On March 31, 2017, the Company converted from an LLC to a C-corporation. Upon the conversion, each outstanding common unit and preferred unit (Note 7) were converted into one share of common stock and preferred stock, respectively. Common units had similar rights and characteristics of common stock issued upon the conversion. In calculating net loss per share, the Company retrospectively applied the effects of the conversion to the number of common units outstanding prior to the conversion. Net loss per share for periods prior to the conversion to a C-corporation refers to net loss per common unit.

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. During periods of income, the Company allocates participating securities a proportional share of income determined by dividing total

weighted average participating securities by the sum of the total weighted average common shares and participating securities (the "two-class method"). The Company's convertible preferred stock participate in any dividends declared by the Company and are therefore considered to be participating securities. Participating securities have the effect of diluting both basic and diluted earnings per share during periods of income. During periods of loss, the Company allocates no loss to participating securities because they have no contractual obligation to share in the losses of the Company. Diluted net loss per share attributable to common stockholders is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock and if-converted methods. For purposes of the diluted net loss per share and per unit attributable to common stockholders calculation, preferred stock, and stock options are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share attributable to common stockholders, as their effect would be anti-dilutive for all periods presented. Therefore, basic and diluted net loss per share and per unit were the same for all periods presented.

Comprehensive Loss

Comprehensive loss is defined as the change in equity during a period from transactions and other events and circumstances from non-owner sources. The Company is required to record all components of comprehensive loss in the Condensed financial statements in the period in which they are recognized. Net loss and other comprehensive loss are reported, net of their related tax effect, to arrive at a comprehensive loss. For the three and nine months ended September 30, 2017 and 2016, comprehensive loss was equal to the net loss.

Recent Accounting Pronouncements

In February 2016, the FASB issued *ASU 2016-02 Leases (Topic 842)* ("ASU 2016-02"), which replaces the existing lease accounting standards. The new standard requires a dual approach for lessee accounting under which a lessee would account for leases as finance (also referred to as capital) leases or operating leases. Both finance leases and operating leases will result in the lessee recognizing a right-of-use asset and corresponding lease liability. For finance leases the lessee would recognize interest expense and amortization of the right-of-use asset and for operating leases the lessee would recognize straight-line total lease expense. ASU 2016-02 is effective for fiscal years, and condensed periods within those years, beginning after December 15, 2018. The Company generally does not finance purchases of equipment but it does lease office and lab facilities. The Company is in the process of evaluating the effect that this ASU will have on its condensed financial statements and related disclosures.

3. Property and Equipment, Net

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

	September 30, 2017 (unaudited)	December 31, 2016
Computer equipment and software	$ 9	$ 8
Laboratory equipment	57	7
Total property and equipment	66	15
Accumulated depreciation and amortization	(11)	(2)
Property and equipment, net	$ 55	13

Depreciation expense was $5 thousand and $9 thousand for the three and nine months ended September 30, 2017, respectively, and $0 and $1 thousand for the three and nine months ended September 30, 2016, respectively.

4. Convertible Promissory Notes

General

On November 16, 2016, the Company entered into a Note Purchase Agreement (the "Note Purchase Agreement") for the issuance of convertible promissory notes (the "Notes") due May 2018. The Notes bore interest at a rate of 6% per annum, which accrued based on a 365-day year. The Notes would have become immediately due and payable in the event of an occurrence of default by the Company. In accordance with their terms, as amended in July 2017, the principal and accrued interest under the Notes were converted into an aggregate of 968,053 shares of preferred stock upon the closing of the Sun Pharma Offering (as defined in Note 7) on August 8, 2017. These shares of preferred stock were then converted into shares of common stock on a 1-to-1 basis upon closing of the IPO.

Conversion Features

The terms of the Notes provided that, in the event the Company sells, merges, consolidates or reorganizes, where the equity owners of the Company own less than 50% of the voting shares post acquisition, then all the outstanding Notes, at the option of the Note holders, either: (i) become immediately due and payable, or (ii) convert into a number of shares of common stock or common units (rounded down to the nearest share), obtained by dividing the outstanding Notes converted by dividing the target valuation of $16.0 million by the fully diluted shares.

Furthermore, in the event that the Company shall issue and sell preferred units in the Company , or if the Company has converted into a corporation, shares of the preferred stock of the Company (in either case, the "Preferred Securities"), to investors for aggregate proceeds to the Company of not less than $5.0 million, then the Notes shall be automatically converted into a number of shares of Preferred Securities equal to the quotient arrived at by dividing the balance of the outstanding Notes so converted by a price per unit or share that is the lesser of: (i) 80% of the per unit or share price of the Preferred Securities issued upon the conversion; or (ii) the target valuation of $16.0 million divided by the fully diluted units or shares immediately prior to closing of the offering which triggered the conversion.

In July 2017, the Notes were amended so that they would also automatically convert upon the closing of an IPO into shares of common stock.

As of September 30, 2017 and December 31, 2016, the Company had outstanding balances related to the Notes of $0 and $1.8 million, respectively. Additionally, as of September 30, 2017 and December 31, 2016, the Company had accrued interest related to the Notes of $0 and $7 thousand, respectively.

Interest expense related to the Notes was $32 thousand and $106 thousand for the three and nine months ended September 30, 2017, respectively, and $0 for each of the three and nine months ended September 30, 2016.

On August 8, 2017, the Notes plus accrued interest were converted into 968,053 shares of preferred stock upon the closing of the Sun Pharma Offering.

On September 22, 2017, all outstanding shares of preferred stock converted into shares of common stock on a 1-to-1 basis upon the closing of the Company's IPO.

5 Related Party Convertible Promissory Notes

On November 14, 2016, the Company issued a convertible promissory note, in the amount of $250 thousand, under the Note Purchase Agreement (Note 4) to a party related to a director of the Company. The terms of the Notes issued to the related party were at an arm's-length and identical to the Notes discussed in Note 4.

On December 27, 2016, the Company issued a convertible promissory note, in the amount of $448 thousand, under the Note Purchase Agreement (Note 4) to an affiliate of two executive officers and directors of the Company. The terms of the Notes issued to the related party were at an arm's-length and identical to the Notes discussed in Note 4.

On May 17, 2017, the Company issued a convertible promissory note, in the amount of $250 thousand, under the Note Purchase Agreement (Note 4) to an affiliate of a director of the Company. The terms of the Notes issued to the related party were at an arm's-length and identical to the Notes issued and discussed in Note 4.

On June 6, 2017, the Company executed a second Note Purchase Agreement (the "Second Note Purchase Agreement") for the issuance of a convertible promissory note to a director of the Company (the "June Note") in the amount of $750 thousand due May 2018. The June Note bore interest at a rate of 6% per annum, which is accrued based on a 365 day year. The June Note would have become immediately due and payable in the event of an occurrence of default by the Company.

Conversion Features

The June Note is convertible into shares of preferred stock automatically upon our closing of a preferred stock financing of at least $5.0 million, or into shares of common stock upon the closing of an initial public offering. The conversion price of the June Note is 80% of the sales price of the preferred stock or 80% of the price at which our common stock is offered to the public in an initial public offering.

As of September 30, 2017 and December 31, 2016, accrued interest on related party convertible promissory notes was $0 and $2 thousand, respectively.

Interest expense related to the related party convertible promissory notes was $12 thousand and $37 thousand for the three and nine months ended September 30, 2017, respectively, and $0 for each of the three and nine months ended September 30, 2016.

On August 8, 2017, upon the closing of the Sun Pharma Offering, the Notes and the June Note plus accrued interest thereon, consisting of $40 thousand, were converted into 360,311 shares of preferred stock. On September 22, 2017, all outstanding shares of preferred stock converted into 2,061,773 shares of common stock on a 1-to-1 basis upon the closing of the IPO.

6 Commitments and Contingencies

Significant Contracts and Agreements

Lease Agreement

In May 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"). In June 2016, the Company entered into an amendment to the 2016 Lease (the "First Amendment to the 2016 Lease"), which amended the timing of the rent payment from one single payment to 17 equal monthly installments. On February 27, 2017, the Company entered into a second amendment to the 2016 Lease, which extended the expiration date of the 2016 Lease to October 31, 2018.

As of September 30, 2017, future minimum operating lease payments and future minimum payments to be received from non-cancelable subleases were as follows (in thousands):

	Operating Leases
2017 (remaining three months)	$ 28
2018	103
2019	—
2020	—
2021	—
Thereafter	—
Future minimum operating lease payments	131
Less: minimum payments to be received from non-cancelable subleases	(23)
Total minimum lease payments, net	$ 108

The Company recorded $20 thousand and $59 thousand in rent expense for the three and nine months ended September 30, 2017, respectively, and recorded $24 thousand and $35 thousand in rent expense for the three and nine months ended September 30, 2016, respectively.

7. Capitalization

Conversion to C-Corporation

On March 31, 2017, the Company converted from an LLC to a C-Corporation. Upon the conversion, all outstanding preferred units and common units were converted on a 1-to-1 basis into shares of preferred stock and common stock, respectively. Following the conversion, the Company had 450,000 authorized shares of preferred stock, $0.00001 par value per share, of which 179,613 shares were issued and outstanding and 45,000,000 authorized shares of common stock, par value $0.00001 per share, of which 3,490,884 shares were issued and outstanding.

Initial Public Offering

On September 22, 2017, the Company completed its initial public offering of 4,554,000 shares of its common stock at a price to the public of $10.00 per share, which includes the sale of 594,000 shares of the Company's common stock pursuant to the underwriters' full exercise of their option to purchase additional shares. The total proceeds from the offering to the Company, net of underwriting discounts and commissions of approximately $3.2 million, were approximately $42.3 million. After deducting offering expenses payable by the Company of approximately $1.5 million, net proceeds to the Company were approximately $40.8 million. Immediately prior to the closing of the IPO, all outstanding shares of the Company's preferred stock converted into 2,061,773 shares of common stock on a 1-to-1 basis.

Shares Outstanding

As of September 30, 2017, 10,237,247 shares of common stock were outstanding. As of September 30, 2017, no shares of preferred stock were outstanding.

As of December 31, 2016, 3,490,884 common units and 179,613 preferred units were outstanding.

Issuance of Preferred Stock and Conversion of Convertible Promissory Notes and Related Party Convertible Promissory Notes

On August 4, 2017, the Company amended its articles of incorporation to authorize 1,500,000 shares of common stock, par value $0.00001 per share, and 1,500,000 shares of preferred stock, par value $0.00001 per share, of which 179,613 shares were designated as Series Seed preferred stock (the "Series Seed Preferred Stock"), 210,000 shares were designated as Series A preferred stock (the "Series A Preferred Stock"), 200,000 shares were designated as Series A-1 preferred stock (the "Series A-1 Preferred Stock") and 30,000 shares were designated as Series A-2 preferred stock (the "Series A-2 Preferred Stock"). The Series Seed Preferred Stock, Series A-1 Preferred Stock and Series A-2 Preferred Stock are collectively known as the preferred stock.

On August 8, 2017, the Company issued 914,107 shares of Series A Preferred Stock to a single investor ("Sun Pharma") at a purchase price of $7.66 per share for aggregate proceeds of approximately $7.0 million (the "Sun Pharma Offering"). Concurrently with the issuance of the Series A Preferred Stock, and in accordance with the conversion features of the Notes (Note 4), all outstanding Notes plus accrued interest thereon were automatically converted into shares of preferred stock.

The following table outlines the conversion on August 8, 2017 of the Notes into shares of preferred stock (in thousands except share and per share amounts**):**

	Principal	Accrued Interest	Total	Conversion Price Per Share(1)	Shares of Series A-1	Shares of Series A-2	Fair Value Date of Conversion	Fair Value Series A-1	Fair Value Series A-2	Loss on Extinguishment of Convertible Promissory Notes
Convertible promissory notes	$ 2,444	$ 72	$2,516	$ 4.14(1)	607,743	—	$ 7.66	$ 4,654	$ —	$ (2,138)
Related party convertible promissory notes	948	32	980	$ 4.14(1)	236,619	—	$ 7.66	1,812	—	(832)
Related party convertible promissory notes —June Note	750	8	758	$ 6.13(2)	—	123,691	$ 7.66	—	947	(189)
Total related party promissory notes	1,698	40	1,738		236,619	123,691		1,812	947	(1,021)
Total	$ 4,142	$ 112	$4,254		844,362	123,691		$ 6,466	$ 947	$ (3,159)

(1) The conversion price was determined by dividing the target valuation of $16 million by the outstanding shares of 3,863,547 immediately prior to the issuance of the Series A on August 8, 2017 (Note 4).
(2) The conversion price was determined to be 80% of the $7.66 sales price per share of the Series A shares issued on August 8, 2017 (Note 5).

Issuance of Common Stock

On August 25, 2017, following the completion of the Sun Pharma Offering, Daniel S. Janney, a member of our board of directors, purchased 130,590 shares of our common stock at the same price per share paid by Sun Pharma, $7.66 per share, through an investment entity owned and controlled by a board member for a total consideration of approximately $1.0 million.

Preferred Units and Preferred Stock

On April 15, 2016, the Company authorized 100 member units and issued 450 member units for aggregate proceeds of $100 thousand. On September 30, 2017, the Company converted all of the member units into 12,771 preferred units at an issue price of $7.83 (the "Original Issue Price") per share plus 3,490,884 common units, and issued an additional 96,345 preferred units at the Original Issue Price for aggregate proceeds of $754 thousand. On December 27, 2016, the Company issued an additional 70,497 preferred units at the Original Issue Price for aggregate proceeds of $552 thousand. On March 31, 2017, all of the preferred units were converted into preferred stock on a 1-to-1 basis. As of September 22, 2017, all of the preferred stock was converted to common stock in connection with the Company's IPO (Note 1). As of September 30, 2017 and December 31, 2016, the Company had balances of preferred units of $0 and $1.4 million, respectively.

The rights, preferences and privileges of the preferred stock consisted of the following:

Conversion. Each share of preferred stock shall be convertible at the option of the holder at any point in time into fully paid and non-assessable shares of common stock. Upon conversion, the preferred stock would be fully settled. Each share of preferred stock was convertible into that number of shares of common stock as determined by dividing the Original Issue Price of such share by the applicable conversion price. As of December 31, 2016, the conversion rate was 1-to-1, but was subject to future adjustments to the conversion price upon the occurrence of certain events including: (i) certain issuances of common stock at a price less than the conversion price in effect on the date of such issuance; and (ii) future stock splits, subdivisions, or combinations of outstanding common stock.

Each share of preferred stock shall automatically be converted into shares of common stock at the conversion price at the time in effect for such series of preferred upon the earlier of: (i) a qualified public offering, as defined in the Certificate of Incorporation; or (ii) the majority vote of the holders of preferred stock on a per share and as-converted to common stock basis.

Voting Rights. The holders of shares of preferred stock are entitled to one vote for each share of preferred stock held at all meetings of stockholders and written actions in lieu of meetings. The Board shall be elected by vote of the common stock and the preferred stock voting together as a single class on an as-converted basis.

Dividends. The holders of the preferred stock are entitled to receive dividends, if and when declared by the Board, and all dividends shall be paid pro rata on the common stock and the preferred stock, without preference, based on the number of shares of the common stock of the holders. From inception through September 30, 2017, no dividends have been declared or paid by the Company.

Liquidation Preference. In the event of any liquidation, dissolution, winding up, consolidation or merger of the Company, the holders of the preferred stock shall be entitled to receive out of the assets of the Company, prior and in preference to any distribution to the holders of common stock, an amount equal to the Original Issue Price per share, plus all declared and unpaid dividends.

The preferred units have the same rights, preferences and privileges as the preferred stock with the exception of conversion rights or liquidation preferences in the event of a merger or consolidation.

Common Units and Common Stock

On September 30, 2016, in connection with the conversion of the member units into preferred units, the Company also issued 3,490,884 common units. On March 31, 2017, in connection with the conversion of the LLC to a C-Corporation, all of the common units were converted, on a 1-to-1 basis, into shares of common stock.

The voting, dividend and liquidation rights of the holders of the common stock are subject to and qualified by the rights, powers and privileges of the holders of the preferred stock and are as follows:

Voting Rights. The holders of shares of common stock are entitled to one vote for each share of common stock held at all meetings of stockholders and written actions in lieu of meetings. The Board shall be elected by vote of the Common Stock and the Preferred stock voting together as a single class on an as-converted basis.

Dividends. The holders of the common stock are entitled to receive dividends, if and when declared by the Board , and all dividends shall be paid pro rata on the common stock and the preferred stock, without preference, based on the number of shares of the common stock of the holders. From inception through September 30, 2017, no dividends have been declared or paid by the Company.

Liquidation Preference. After payment to the holders of shares of preferred stock of their liquidation preferences, the holders of the common stock are entitled to share ratably in the Company's assets available for distribution to stockholders, in the event of any voluntary or involuntary liquidation, dissolution, winding up, consolidation or merger of the Company or upon the occurrence of a deemed liquidation event.

8. Significant Agreements

Clinical Supply Agreement

In December 2016, the Company entered into a product manufacturing and clinical supply agreement with a Contract Manufacturing Organization (the "CMO"). The product manufacturing and clinical supply agreement provides the terms and conditions under which the CMO will formulate, fill, inspect, package, label and test our lead product, KB103 for clinical supply. The Company is obligated to pay the CMO for each batch of KB103 manufactured. Additionally, certain raw materials, supplies, outsourced testing and other services for the purposes of batch production will be invoiced separately by the CMO. The estimated remaining commitment under this agreement for the manufacturing of our drug product is approximately $1.7 million. The Company is also responsible for the payment of a monthly service fee for project management services for the duration of the arrangement. The Company has incurred expenses under this agreement of $483 thousand and $643 thousand for the three and nine months ended September 30, 2017, respectively, and $0 for the three and nine months ended September 30, 2016.

9. Stock-Based Compensation

On October 1, 2016, the Board of Managers adopted the 2016 Equity Incentive Plan (the "2016 Plan"), which authorizes the issuance of up to 189,472 incentive units to purchase common units.

The 2016 Plan provided for the issuance of incentive units to employees, members of the Board of Managers, and consultants of the Company. The incentive units generally expire ten years following the date of grant. The incentive units typically vest over a period of four years, but vesting provisions can vary by award based on the discretion of the Board of Managers. Incentive units to purchase common units carry an exercise price equal to the estimated fair value of the Company's common units on the date of grant. Generally incentive units to purchase common units of the Company are exercised by payment of the exercise price in cash. Upon the termination of service, except by death or disability, of a holder of incentive units awarded under the 2016 Plan, all unvested units are forfeited and vested incentive units may be exercised within three months of termination by the holder. Common units issued as a result of awards under the 2016 Plan may be subject to repurchase provisions as designated in each individual award agreement.

On March 31, 2017, the Board adopted the 2017 Stock Incentive Plan (the "2017 Plan") which authorized the issuance of up to 193,050 shares of the Company's common stock under the plan. Commensurate with the opening of the 2017 Plan, all 113,683 outstanding incentive units granted under the 2016 Plan were converted into 113,683 options to purchase the Company's common stock under the 2017 Plan. Upon the adoption of the 2017 Plan, the 2016 Plan terminated and there were no incentive units outstanding.

The 2017 Plan provided for the issuance of stock options, restricted stock awards and unrestricted stock awards to employees, members of the Board, and consultants of the Company. The Company has not granted restricted or unrestricted stock awards under the 2017 Plan since its inception. Options generally expire ten years following the date of grant. Options typically vest over a period of four years, but vesting provisions can vary by award based on the discretion of the Board. Options to purchase common stock carry an exercise price equal to the estimated fair value of the Company's common stock on the date of grant. Generally options to purchase shares of the Company's common stock are exercised by payment of the exercise price in cash. Upon the termination of service, except by death or disability, of a holder of stock options awarded under the 2017 Plan, all unvested options are forfeited and vested options may be exercised within three months of termination by the holder. Shares of common stock issued as a result of awards under the 2017 Plan may be subject to repurchase provisions as designated in each individual award agreement.

Shares of common stock underlying awards previously issued under the 2017 Plan which are reacquired by the Company, withheld by the Company in payment of the purchase price, exercise price, or withholding taxes; expired; cancelled due to forfeiture, or otherwise terminated other than by exercise, are added to the number of shares of common stock available for issuance under the 2017 Plan. Shares available for issuance under the 2017 Plan may be authorized but unissued shares of the Company's common stock or common stock reacquired by the Company and held in treasury. The 2017 Plan terminated upon the adoption of the 2017 IPO Plan (as defined below).

On September 5, 2017, the Board approved the establishment of the Krystal Biotech, Inc. 2017 IPO Plan (the "2017 IPO Plan"), which was adopted prior to the effectiveness of our registration statement. Under the 2017 IPO Plan, the Company may grant incentive stock options, non-qualified stock options, stock appreciation rights, dividend equivalent rights, restricted stock, and stock grants to purchase up to 900,000 shares of the Company's Common Stock.

The Company granted 71,649 and 142,105 stock options during the nine months ended September 30, 2017 and through December 31, 2016, respectively, to employees, consultants and board members, which are included in the following table. The options vest ratably over a four-year period, and have a life of ten years. Stock options issued to non-employees are accounted for using the fair value method of accounting, and are periodically revalued as the options vest, and are recognized as expense over the related service period.

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

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (In thousands) (1)
Outstanding at January 1, 2016	—			
Granted	142,105	$ 2.46	9.7	$ —
Exercised	—			
Cancelled or forfeited	—			
Outstanding at December 31, 2016	142,105	$ 2.46	9.7	$ 338
Granted (unaudited)	71,649	$ 6.22		
Exercised (unaudited)	—	—		
Cancelled or forfeited (unaudited)	(28,422)	$ 2.46		
Outstanding at September 30, 2017 (unaudited)	185,332	$ 3.91	9.3	$ 1,122
Exercisable at September 30, 2017 (unaudited)	18,945	$ 2.46	8.9	$ 142
Vested at September 30, 2017 (unaudited)	18,945	$ 2.46	8.9	$ 142

(1) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in the money at September 30, 2017 (unaudited) and December 31, 2016.

As of September 30, 2017, 900,000 shares of common stock were available for future grants under the 2017 IPO Plan.

The weighted-average grant-date fair value of options granted to employees during the nine months ended September 30, 2017 was $6.42.

Stock-based compensation expense for the three and nine months ended September 30, 2017 and 2016 relates solely to stock options granted under the 2017 Plan. The Company has recorded aggregate stock-based compensation expense related to the issuance of stock option awards to employees and non-employees in the statement of operations for the three and nine months ended September 30, 2017 and 2016 as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
	(unaudited)		(unaudited)	
Research and development	$ 40	$ 4	$ 107	$ 7
General and administrative	14	—	81	—
Total stock-based compensation	$ 54	$ 4	$ 188	$ 7

Stock Options Granted to Employees. For the three and nine months ended September 30, 2017, the Company recorded $47 thousand and $78 thousand, respectively, of stock-based compensation expense related to employees' stock options. For the three and nine months September 30, 2016, the Company had no employees. The fair value of options granted to employees 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, 2017:

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
	(unaudited)		(unaudited)	
Expected stock price volatility	80%	—	80%	—
Expected term of the award (years)	6.25	—	6.25	—
Risk-free interest rate	1.92%	—	1.93%	—
Exercise price	$ 8.27	—	$ 6.22	—
Estimated fair value of its Common Stock on the measurement date	$ 8.27	—	$ 8.46	—

There was $671 thousand of unrecognized stock-based compensation expense related to employees' awards that is expected to be recognized over a weighted-average period of 3.26 years as of September 30, 2017.

Stock Options Granted to Non-Employees. Stock-based compensation expense related to stock options granted to non-employees is recognized as the stock options are earned. The Company believes that the estimated fair value of the stock options is more readily measurable than the fair value of the services rendered. For the three and nine months ended September 30, 2017, and three and nine months ended September 30, 2016, the Company recorded $7 thousand, $110 thousand, $4 thousand and $7 thousand, respectively, of stock-based compensation expense related to non-employees' stock options.

The Company used the following weighted-average assumptions in estimating non-employees stock-based compensation expense:

	Three and nine months ended September 30, 2016
Expected stock price volatility	80%
Expected term of the award (years)	6.25
Risk-free interest rate	1.97%
Exercise price	$ 2.46
Estimated fair value of its Common Stock on the measurement date	$ 2.46

There were no options granted to non-employees in the nine months ended September 30, 2017.

10. Related Party Transactions

Convertible Promissory Notes

As of September 30, 2017 and December 31, 2016, the Company had balances of $0 and $698 thousand in related party convertible promissory notes, respectively (see Note 5).

Preferred Units

As of September 30, 2017 and December 31, 2016, the Company had outstanding balances of $0 and $1.4 million of preferred units, respectively. The preferred units as of December 31, 2016 were all held by the Chief Executive Officer and Chief Operating Officer of the Company as disclosed in Note 7.

Sale of Common Stock

On August 25, 2017, the Company closed the sale of 130,590 shares of Common Stock with an affiliate of a member of the Board for aggregate proceeds of $1.0 million.

In connection with our IPO, our Chief Executive Officer and Chief Operating Officer of the Company each purchased 100,000 shares of common stock at the offering price of $10 per share.

11. Subsequent Events

On November 1, 2017, the Company entered into a stock purchase agreement (the "Agreement") with the Epidermolysis Bullosa Medical Research Foundation, a California nonprofit 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 has agreed to issue and sell, and the Purchasers have agreed to purchase, an aggregate of 70,000 shares of the Company's common stock, par value $0.00001 per share, for a purchase price of $11.00 per share, resulting in aggregate gross proceeds to the Company of $770,000 (the "Transaction"). The offer, sale and issuance of the shares of the Company are exempt from registration pursuant to Rule 506 of Regulation D and Section 4(a)(2) of the Securities Act of 1933, as amended. Pursuant to the Agreement, the Purchasers have agreed they are acquiring the securities for investment only and not with a view to or for sale in connection with any distribution thereof and appropriate legends are to be affixed to the securities to be issued in conjunction with the Agreement. The Purchasers are subject to a six month lock-up, and the shares purchased under the terms of the Agreement will be subject to restrictions on selling, transferring or otherwise disposing of such shares. The Transaction closed on November 2, 2017.

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 Condensed financial statements and related notes included in Item 1 of Part I of this Quarterly Report on Form 10-Q and with the audited Condensed financial statements and the related notes included in our Prospectus on Form S-1 for the fiscal year ended December 31, 2016, as amended, and filed with the Securities and Exchange Commission, or the SEC on September 19, 2017.

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, and Section 21E of the Securities Exchange Act of 1934, as amended. All statements contained in this Quarterly Report on Form 10-Q other than statements of historical fact, including statements regarding our future results of operations and financial position, our business strategy and plans, and our objectives for future operations, are forward-looking statements. The words "believe," "may," "will," "potentially," "estimate," "continue," "anticipate," "intend," "could," "would," "project," "plan," "expect" and the negative and plural forms of these words and similar expressions are intended to identify forward-looking statements.

Overview

We are a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from dermatological diseases. We have developed a proprietary gene therapy platform, which we refer to as the Skin TARgeted Delivery platform, or STAR-D platform, that consists of an engineered patent pending viral vector based on herpes simplex virus 1, or HSV-1, and skin-optimized gene transfer technology, to develop off-the-shelf treatments for dermatological diseases for which we believe there are no known effective treatments. We are initially using our STAR-D platform to develop treatments for rare or orphan dermatological indications caused by the absence of or a mutation in a single gene, and plan to leverage our platform in the future to expand our pipeline to include other dermatological indications.

Our lead product candidate, KB103, is currently in preclinical development and seeks to use gene therapy to treat dystrophic epidermolysis bullosa, or DEB, a rare and severe genetic 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 protein type VII collagen, or COL7, that forms anchoring fibrils that bind the dermis to the epidermis. 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. Based on information from DEBRA International, a worldwide alliance of patient support groups for epidermolysis bullosa, or EB, of which DEB is a subset, we believe there may be as many as 125,000 patients worldwide who suffer from DEB. We estimate that there are 3,200 to 3,500 diagnosed DEB patients in the European Union, United States, Japan and Canada.

Our company was organized in December 2015 in the State of California and commenced operations on April 15, 2016. On March 31, 2017, we converted from a California limited liability company to a Delaware C-corporation, and changed our name from Krystal Biotech, LLC to Krystal Biotech, Inc. To date, our operations have been limited to organizing and staffing our company, developing our proprietary STAR-D platform, identifying potential product candidates and undertaking preclinical studies and preparing for clinical trials of our product candidates.

On September 19, 2017, the Company's registration statement on Form S-1 (File No. 333-220085) relating to the IPO was declared effective by the SEC. On September 22, 2017, the Company completed its IPO of 4,554,000 shares of its common stock at a price to the public of $10.00 per share, which includes the sale of 594,000 shares of the Company's common stock pursuant to the underwriters' full exercise of their option to purchase additional shares. Total proceeds from the offering to the Company, net of underwriting discounts and commissions of $3.2 million, were $42.3 million. After deducting offering expenses payable by the Company of $1.5 million, net proceeds to the Company were $40.8 million. At September 30, 2017, our cash balance was approximately $52.6 million.

On November 1, 2017, the Company entered into a stock purchase agreement (the "Agreement") with the Epidermolysis Bullosa Medical Research Foundation, a California nonprofit 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 has agreed to issue and sell, and the Purchasers have agreed to purchase, an aggregate of 70,000 shares of the Company's common stock, par value $0.00001 per share, for a purchase price of $11.00 per share, resulting in aggregate gross proceeds to the Company of $770,000.

On November 2, 2017, the U.S. Food & Drug Administration (FDA) granted Orphan Drug Designation to the Company's lead product candidate, KB103, for the treatment of dystrophic epidermolysis bullosa ("DEB"). The FDA's Office of Orphan Drug Products grants orphan drug designation to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the United States. Orphan drug designation will may allow Krystal Biotech to be eligible for a seven-year period of U.S. Marketing exclusivity upon approval of KB103, tax credits for certain clinical research costs, and a waiver of the Prescription Drug User Fee Act (PDUFA) filing fees, subject to certain conditions.

On November 3, 2017, the Office of Science Policy (OSP) at the National Institutes of Health (NIH) indicated that the Company's Phase 1/2 protocol for KB103 has completed the NIH Recombinant DNA Advisory Committee (RAC) protocol registration process.

Since operations began, we have incurred operating losses. Our net losses were $5.3 million and $6.5 million for the three and nine months ended September 30, 2017, and $322 thousand and $523 thousand for the three and nine months ended September 30, 2016, respectively. At September 30, 2017, we had accumulated a deficit of $7.7 million. 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 will need to generate significant revenue to achieve profitability, and we may never generate revenue or enough revenue to achieve profitability.

Costs related to clinical trials can be unpredictable and therefore there can be no guarantee that we will have sufficient proceeds from our IPO and from other sources to fund our planned preclinical and clinical studies or our operations. Our funds may not be sufficient to enable us to conduct pivotal clinical trials for, seek marketing approval for or commercially launch KB103 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, or at all. Our failure to raise capital when needed would have a negative effect on our financial condition and our ability to pursue our 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, or CMOs, consultants and other vendors that conduct our preclinical activities;
- costs of acquiring, developing and manufacturing clinical trial materials and lab supplies; and
- facility costs, depreciation and other expenses, which include direct expenses for rent and maintenance of facilities and other supplies.

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 manufacture 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 conduct our ongoing preclinical trials and our planned Phase 1/2 clinical trial for KB103. Due to the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration, costs and timing of this clinical trial, and, as a result, the actual costs to complete this planned clinical trial may exceed the expected costs.

General and Administrative Expenses

General and administrative expenses consist principally of professional fees associated with corporate and intellectual property legal expenses, consulting and accounting services and facility-related costs. 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 Expense, Net

Interest expense, net consists primarily of interest expense incurred due to the beneficial conversion feature upon conversion of promissory notes to shares of preferred stock, and to a lesser degree due to interest expense on our convertible promissory notes before their conversion to shares of preferred stock, offset by interest earned on our cash.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial position and results of operations is based on our Condensed financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of Condensed financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the amounts reported in the Condensed financial statements and accompanying notes. On an ongoing basis, we evaluate estimates which include, but are not limited to, estimates related to contract manufacturing prepayments and accruals, stock-based compensation expense, and reported amounts of expenses during the reported period. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances. Actual results may differ materially from those estimates or assumptions.

During the quarter ended September 30, 2017, there were no significant changes to the items that we disclosed as our critical accounting policies and estimates in Note 2 to our consolidated Condensed financial statements for the year ended December 31, 2016 contained in our final prospectus filed with the SEC on September 19, 2017.

Results of Operations

Three Months Ended September 30, 2017 and September 30, 2016

(in thousands)	Three Months Ended September 30, 2017 (unaudited)	2016 (unaudited)
Expenses		
Research and development	$ 1,355	241
General and administrative	738	81
Total operating expenses	2,093	322
Loss from operations	(2,093)	(322)
Other Expense		
Interest expense, net	(3,180)	—
Total other expense	(3,180)	—
Net loss	$ (5,273)	$ (322)

Research and Development Expenses

Research and development expenses increased $1.1 million thousand in the three months ended September 30, 2017 as compared to the three months ended September 30, 2016. Higher research and development expenses was due largely to increases in professional services related to outsourced manufacturing and in-vivo studies of $867 thousand, payroll, employee benefits and stock-based compensation of $192 thousand, lab supplies of $27 thousand, and other research and development expenses of $28 thousand.

General and Administrative Expenses

General and administrative expenses increased $657 thousand in the three months ended September 30, 2017 as compared to the three months ended September 30, 2016. Higher general and administrative spending was due largely to increases in legal and professional services of $472 thousand, payroll, employee benefits and stock-based compensation costs of $123 thousand, franchise taxes of $37 thousand, insurance expenses of $17 thousand as a result of being a public company, and other administrative costs of $8 thousand.

Interest Expense, Net

Interest expense, net, was $3.2 million primarily due to the beneficial conversion feature upon conversion of promissory notes to shares of preferred stock. There was no interest expense for the three months ended September 30, 2016.

Nine Months Ended September 30, 2017 and September 30, 2016

(in thousands)	Nine Months Ended September 30, 2017	2016
	(unaudited)	
Expenses		
Research and development	$ 2,120	$ 339
General and administrative	1,154	184
Total operating expenses	3,274	523
Loss from operations	(3,274)	(523)
Other Expense		
Interest expense, net	(3,253)	—
Total other expense	(3,253)	—
Net loss	$ (6,527)	$ (523)

Research and Development Expenses

Research and development expenses increased $1.8 million in the nine months ended September 30, 2017 as compared to the nine months ended September 30, 2016. Higher research and development spending was due largely to increases in professional services related to outsourced manufacturing and in-vivo studies of $1.2 million, lab supplies of $157 thousand, payroll and employee benefits of $210 thousand, stock-based compensation of $107 thousand, and other research and development expenses of $73 thousand.

General and Administrative Expenses

General and administrative expenses increased $970 thousand in the nine months ended September 30, 2017 as compared to the nine months ended September 30, 2016. Higher general and administrative spending was due largely to increases in legal and professional services of $631 thousand, payroll and employee benefits of $128 thousand, stock-based compensation costs of $74 thousand, travel and related costs of $66 thousand and other administrative costs of $71 thousand.

Interest Expense, Net

Interest expense, net was $3.3 million for the nine months ended September 30, 2017 related to $3.2 million in interest expense incurred due to the beneficial conversion feature upon conversion of promissory notes to shares of preferred stock, interest expense of our convertible promissory notes of $105 thousand partially offset by $10 thousand in interest income earned from our cash proceeds. There was no interest expense, net for the nine months ended September 30, 2016.

Liquidity and Capital Resources

Overview

As of September 30, 2017 and December 31, 2016, the Company had accumulated deficits since inception of $7.7 million and $1.2 million, respectively. On September 22, 2017, the Company received net proceeds of approximately $40.8 million from its initial public offering. The Company believes that its cash of approximately $52.6 million as of September 30, 2017 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 or obtain financing from other sources, such as partnerships. 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 assurances, however, that additional funding will be available on terms acceptable to the Company, or at all.

In August 2017, we closed the sale of preferred stock to a single investor for aggregate proceeds of $7.0 million, and the sale of 130,590 shares of our common stock with a party related to a director of our board for aggregate proceeds of $1.0 million.

Prior to August 2017, we had received $1.4 million in gross proceeds from the issuance of equity securities and $4.1 million in gross proceeds from debt financings.

Debt Financings

On November 16, 2016, we executed a note purchase agreement under which convertible promissory notes were issued. Each note bore interest at a rate of 6% per annum, which accrued based on a 365 day year and matured on May 14, 2018, unless sooner paid or converted. As of December 31, 2016 and the outstanding principal balance of the notes was $1.8 million.

Commensurate with the sale and issuance of the preferred stock on August 8, 2017, the convertible promissory notes were converted into 968,053 shares of preferred stock (inclusive of accrued interest thereon), in accordance with the conversion features of the notes. Upon the closing of the IPO, all shares of preferred stock, including previously converted promissory notes, converted into 2,061,773 shares of common stock with a 1-to-1 conversion ratio.

Operating Capital Requirements

Our primary uses of capital are, and we expect will continue to be for the near future, compensation and related expenses, manufacturing costs for preclinical and clinical materials, third party clinical trial research and development services, laboratory and related supplies, clinical costs, legal and other regulatory expenses and general overhead costs.

We believe that our available funds will be sufficient to enable us to obtain clinical data from our planned Phase 1/2 clinical trial for KB103. We expect that these funds will not be sufficient to enable us to seek marketing approval for or commercialize any of our product candidates.

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 timing and costs of our ongoing planned Phase 1/2 clinical trial for KB103;
- the progress, timing and costs of manufacturing of KB103 for planned clinical trials;
- the initiation, progress, timing, costs and results of preclinical studies and clinical trials for our other product candidates and potential product candidates;
- the outcome, timing and costs of seeking regulatory approvals;
- the costs of commercialization activities for KB103 and other product candidates if we receive marketing approval, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue received from commercial sales of our 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, 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;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and
- the extent to which we in-license or acquire other products and technologies.

We expect that we will need to obtain substantial additional funding in order to receive regulatory approval and to commercialize KB103 or any other product candidates. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our existing stockholders may be materially diluted and the terms of these securities could include liquidation or other preferences that could adversely affect the rights of our existing stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely affect our ability to conduct our business. If we are unable to raise capital when needed or on attractive terms, we could be forced to significantly delay, scale back or discontinue the development or commercialization of KB103 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 KB103 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,	
	2017	2016
	(unaudited)	
Net cash used in operating activities	$ (1,763)	$ (450)
Net cash used in investing activities	(51)	(6)
Net cash provided by financing activities	52,461	854
Net increase in cash	$ 50,647	$ 398

Operating Activities

Net cash used in operating activities was $1.8 million for the nine months ended September 30, 2017 consisting primarily of a net loss of $6.6 million adjusted for non-cash items including interest expense incurred of $3.3 million due to the beneficial conversion feature upon conversion of promissory notes to shares of preferred stock, depreciation and stock-based compensation expense of $197 thousand and a net decrease in operating assets and liabilities of approximately $1.3 million.

Net cash used by operating activities was $450 thousand for the nine months ended September 30, 2016, which consisted primarily of a net loss of $523 thousand adjusted for non-cash items, including depreciation and stock-based compensation expense of $7 thousand, and a net decrease in operating assets and liabilities of $65 thousand.

Investing Activities

During the nine months ended September 30, 2017 and 2016, our investing activities used net cash of approximately $51 thousand and $6 thousand, respectively, primarily from the purchase of computer and laboratory equipment.

Financing Activities

Net cash provided by financing activities was $52.5 million for the nine months ended September 30, 2017, which consisted of total proceeds of approximately $43.2 million including proceeds from the sale of common stock in our IPO on September 22, 2017 net of underwriting discounts and commissions of approximately $3.2 million, less offering expenses paid by the Company of $190 thousand, $7.0 million in proceeds from the sale of Series A Preferred Stock to a single investor, $2.3 million in proceeds from the issuance of convertible promissory notes and $1.0 million in proceeds from the sale of shares of common stock to an affiliate of a member of the Board.

Net cash provided by financing activities was $854 thousand for the nine months ended September 30, 2016, which consisted of $100 thousand from the issuance of common units and $754 thousand from the issuance of preferred units.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements.

Contractual Obligations

Our future contractual obligations were reported in our Registration Statement Form S-1 (File No. 333-220085) relating to the IPO on September 19, 2017. There have been no material changes from the contractual obligations as previously disclosed in our Registration Statement.

JOBS Act Accounting Election

We are an emerging growth company, as defined in the Jumpstart Our Business startups Act of 2012, or 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 of $52.6 million at September 30, 2017, which consist primarily of bank deposits and money market funds. The investments in these financial instruments are made in accordance with an investment policy which specifies the categories, allocations and ratings of securities we may consider for investment. The primary objective of our investment activities is to preserve principal while at the same time maximizing the income we receive without significantly increasing risk. Some of the financial instruments in which we invest could be subject to market risk. This means that a change in prevailing interest rates may cause the value of the instruments to fluctuate. For example, if we purchase a security that was issued with a fixed interest rate and the prevailing interest rate later rises, the value of that security will probably decline. To minimize this risk, we intend to maintain a portfolio which may include cash, cash equivalents and 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 has significant risk of default or illiquidity. While we believe our cash account does 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 is recorded at fair value.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our Chief Executive Officer and our Chief Financial 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 Financial 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 were no changes in our internal control over financial reporting during the three month period covered by this Quarterly Report on Form 10-Q that have materially affected or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

We currently are not a party to any material litigation or other material legal proceedings. We may, from time to time, be subject to legal proceedings and claims arising from the normal course of business activities.

ITEM 1A. RISK FACTORS

RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL CAPITAL

We have never generated revenue and may never be profitable.

Since inception, we have incurred recurring losses and negative cash flows from operations and, at September 30, 2017, we have an accumulated deficit of $7.7 million. Our ability to achieve profitability depends on our ability to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, KB103 and any additional product candidates that we may pursue in the future. We do not anticipate generating revenues from product sales for the next several years, if ever. We have devoted substantially all of our efforts to research and development of our gene therapy product candidate, KB103, 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 preclinical and clinical development of KB103, including our planned clinical trials;
- initiate additional clinical trials and preclinical studies for any additional product candidates that we may pursue in the future;

- prepare our biologics license application, or BLA, and marketing authorization application for KB103;
- manufacture current good manufacturing practices, or cGMP, material for clinical trials or potential commercial sales;
- establish and validate a commercial-scale cGMP manufacturing facility;
- 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 KB103 in the European Union 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 preclinical testing and clinical trials of KB103, 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. In addition, if we were required to discontinue development of KB103, if KB103 does not receive regulatory approval, if we do not obtain our targeted indications for KB103 or if KB103 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 KB103 product candidate 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 two product candidates, KB103 and KB104, and we may never develop, acquire or in-license additional product candidates. We may never succeed in any or all of 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 U.S. Food and Drug Administration, or FDA, or the European Medicines Agency, or 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 KB103, our expenses could increase and revenue could be further delayed.

We will need to raise additional funding in order to receive approval for KB103 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.

In order to complete the process of obtaining regulatory approval for KB103 and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize KB103, if approved, we will require substantial additional funding. In addition, if we obtain marketing approval for KB103, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. We anticipate that we will need additional funding to complete the development of KB103 and any future product candidates and to commercialize any such approved products.

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

- the progress and results of our planned clinical trials of KB103;
- the scope, progress, results and costs of drug discovery, laboratory testing, manufacturing, preclinical development and clinical trials for any other product candidates that we may pursue in the future, if any;
- the costs, timing and outcome of regulatory review of KB103 and any other product candidates we may develop;
- the costs of establishing and maintaining our own commercial-scale cGMP manufacturing facility;
- 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 KB103 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;
- revenue, if any, received from commercial sale of KB103 or other product candidates, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- 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.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, 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 of 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.

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 KB103, have been limited to organizing and staffing our company, business planning, raising capital, developing our STAR-D platform and related technologies, identifying KB103 as a potential gene therapy product candidate and undertaking preclinical trials of KB103. We have not yet demonstrated the ability to complete clinical trials of KB103 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 or viability may not be as accurate as they could be if we had more experience developing gene therapy products.

We do not currently have the ability to perform the sales, marketing and manufacturing functions necessary for the production and sale of KB103 on a commercial scale. Our lead product candidate, KB103, will be required to undergo significant clinical trials before it can be commercialized, if at all. The successful commercialization of KB103 will require us to perform a variety of functions, including:

- clinical development of KB103;
- obtaining required regulatory approvals;
- formulating and manufacturing product candidates; and
- conducting sales and marketing activities.

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

We are early in our development efforts and our lead product candidate, KB103, is still in preclinical development. If we are unable to advance KB103 to clinical trials, obtain regulatory approval and ultimately commercialize KB103, or if we experience significant delays in doing so, our business will be materially harmed.

We are early in our development efforts and KB103 is still in preclinical development. Pending additional regulatory approvals, we plan to initiate a Phase 1/2 clinical trial in the first quarter of 2018. The development and commercialization of KB103 or any other product candidate we may develop is subject to many uncertainties, including the following:

- successful completion of additional preclinical studies and successful enrollment and completion of clinical trials;
- an effective investigational new drug application, or IND, and clinical trial authorizations, or CTA, that allow us to commence our planned clinical trials for KB103;
- positive results from our planned clinical trials;
- receipt of regulatory approvals from applicable regulatory authorities;
- maintenance of our existing arrangements with third-party manufacturers for clinical supply and successful development of our internal manufacturing processes on an ongoing basis;
- commercial launch of KB103, if and when approved, whether alone or in collaboration with others;
- acceptance of KB103, if and when approved, by patients, the medical community and third-party payors;
- enforcement and defense of intellectual property rights and claims; and
- maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize KB103, which would materially harm our business. If we do not receive regulatory approvals for KB103, our business, financial condition, results of operations and prospects could be materially and adversely affected.

We have not tested KB103 in clinical trials. Success in early preclinical studies may not be indicative of results obtained in later preclinical studies and clinical trials.

KB103 has never been evaluated in human clinical trials, and we may experience unexpected or adverse results in the future. We will be required to demonstrate through adequate and well-controlled clinical trials that KB103 is safe for humans and effective for indicated uses before we can seek regulatory approvals for commercial sale.

The positive results we have observed for KB103 in preclinical trials may not be predictive of outcomes in our future clinical trials. KB103, or other product candidates, may also fail to show the desired safety and efficacy in later stages of clinical development even if they successfully advance through initial clinical trials. The clinical trial process may fail to demonstrate that KB103 is safe for humans and effective for indicated uses, which may cause us to abandon KB103, which is currently our lead product candidate.

Many companies in the biotechnology industry have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and there is a high failure rate for product candidates proceeding through clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. Regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development, failure to perform in accordance with FDA good clinical practices or applicable regulatory guidelines in the EU and other countries, selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data, or changes in regulatory requirements and guidance that require amending or submitting new clinical protocols. In addition, the design of a clinical trial can determine whether its results will support approval of a product, and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We cannot be certain that we will not face these or similar setbacks.

We may find it difficult to enroll an adequate number of patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of KB103.

Identifying and qualifying patients to participate in clinical trials of KB103 is critical to our success. The timing of our clinical trials depends on our ability to recruit an adequate number of patients to participate as well as completion of required follow-up periods. If patients are unwilling to participate in our gene therapy studies because of competitive clinical trials for similar patient populations, negative publicity from adverse events related to the biotechnology or gene therapy fields or for other reasons, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of KB103 may be delayed. These delays could result in increased costs, delays in advancing KB103, delays in testing the effectiveness of KB103 or termination of clinical trials altogether.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize KB103 and the approval may be for a more narrow indication than we seek.

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if KB103 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 Risk Evaluation and Mitigation Strategy, or REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of KB103. Any of the foregoing scenarios could materially harm the commercial prospects for KB103 and materially and adversely affect our business, financial condition, results of operations and prospects.

KB103 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. To date, only one gene therapy product, Novartis' Kymriah, has received marketing approval by the FDA, and only two gene therapy products, uniQure N.V.'s Glybera® and GlaxoSmithKline's Strimvelis™, have received marketing authorization from the European Commission. 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 Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the United States National Institutes of Health, or the NIH, also are potentially subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC; however, the NIH recently announced that the RAC will only publicly review clinical trials if the trials cannot be evaluated by standard oversight bodies and pose unusual risks. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution to conduct a clinical trial, that institution's institutional biosafety committee as well as its institutional review board, or IRB, would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of our product candidates. 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 KB103 or future product candidates or lead to significant post-approval limitations or restrictions. As we advance KB103, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of KB103. 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.

KB103 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. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which, while not necessarily adverse to the patient's health, could substantially limit the effectiveness of the treatment. In previous clinical trials involving vectors derived from adeno-associated virus for gene therapy, some subjects experienced the development of a T-cell response, whereby after the vector is within the target cell, the cellular immune response system triggers the removal of transduced cells by activated T-cells. If our vectors demonstrate a similar effect we may decide or be required to halt or delay further clinical development of KB103.

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, KB103 for any or all targeted indications. Even if we are able to 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 KB103, 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 KB103 receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by KB103, 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 KB103 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 KB103, 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 KB103 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 KB103 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;
- 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 institutional review board, or 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, or 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.

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, with only one gene therapy product approved to date in the United States and only two gene therapy products approved to date in the European Union. 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.

In addition, our success will depend upon physicians who specialize in the treatment of DEB prescribing treatments that involve the use of KB103 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 KB103 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 KB103, stricter labeling requirements for KB103 if approved and a decrease in demand for KB103.

If the market opportunities for KB103 or our future product candidates are smaller than we believe they are, our product revenues may be adversely affected and our business may suffer.

We are currently focusing our research and product development efforts on our KB103 treatment 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 KB103, 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 European Union and elsewhere may turn out to be lower than expected or these patients may not be otherwise amenable to treatment with KB103 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 KB103 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 KB103 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 KB103. Even with the requisite approvals from the FDA in the United States, the EMA in the European Union and other regulatory authorities internationally, the commercial success of KB103 will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and KB103 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, in particular, KB103, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of KB103 as demonstrated in clinical trials;
- the efficacy, potential and perceived advantages of KB103 over alternative treatments;
- the cost of KB103 relative to alternative treatments;
- the clinical indications for which KB103 is approved by the FDA or the European Commission;
- patient awareness of, and willingness to seek, genotyping;
- 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.

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 KB103 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 KB103 in the European Union, 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 European Union 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 KB103 or our future product candidates will be harmed and our business, financial condition, results of operations and prospects will be adversely affected.

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. For example, we currently do not have a full-time chief financial officer or principal accounting officer in-house, and rely on professional service providers for these functions. We expect to experience a period of significant expansion in headcount, facilities, infrastructure and overhead as we mature and to meet our new reporting requirements under the Securities Exchange Act of 1934, as amended. 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 KB103, 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 KB103 may also be subject to a REMS, 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. FDA guidance advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years, and our current and each of our proposed clinical trials for KB103 includes a 15 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 KB103 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 KB103 and adversely affect our business, financial condition, results of operations and prospects.

In addition, 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 KB103. 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.

We may be unable to obtain orphan drug exclusivity for KB103 or any other future product candidate. If our competitors are able to obtain orphan drug exclusivity for products that constitute the same drug and treat the same indications as KB103 before us, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

We plan to seek an orphan drug designation from the FDA for KB103. Regulatory authorities in some jurisdictions, including the United States and the European Union, 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 European Union, 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 European Union. 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 European Union 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 European Commission 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 European Union. The exclusivity period in the European Union can be reduced to nine 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 if we obtain orphan drug exclusivity for KB103, 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 European Union, 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 similar to 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 sufficient quantities of orphan medicinal product.

Breakthrough 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.

We may, in the future, apply for breakthrough therapy designation or Fast Track designation in the United States for our product candidates. We plan to apply for breakthrough therapy designation in the first quarter of 2017 and Fast Track designation in the second quarter of 2018 for KB103 and we have been granted rare pediatric disease designation for KB103. Each of these designations is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for breakthrough therapy designation, Fast Track designation or rare pediatric disease designation, the FDA may disagree. In any event, 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 procedures and does not assure ultimate approval by the FDA.

A breakthrough 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 or condition and preliminary clinical evidence indicates that such product candidate may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. Drugs designated as breakthrough 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 if we do receive 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. In December 2016, we received the designation of "rare pediatric disease" for KB103 and conditional designation of our marketing application as a "rare pediatric disease product application," which, if granted, could qualify us to receive a Rare Pediatric Priority Review Voucher. According to the FDA website, a Rare Pediatric Priority Review Voucher can be redeemed to receive a priority review of a subsequent marketing application for a different product.

There is no assurance we will receive 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 KB103, we may not experience a faster development process, review or approval for a subsequent marketing application.

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

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 KB103, 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 or not 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 KB103.

At this time, there are no known FDA or EMA approved treatments for DEB, or any approved gene therapy treatment for dermatological indications, generally. However, 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 KB103 uneconomical or obsolete, and we may not be successful in marketing KB103 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.

Delays in obtaining regulatory approvals of the process and facilities needed to manufacture KB103 or disruptions in our manufacturing process may delay or disrupt our product development and commercialization efforts.

Before we can begin to commercially manufacture KB103, whether in a third-party facility or in our own facility, once established, we must obtain regulatory approval from FDA for our manufacturing process and facility. A manufacturing authorization must also be obtained from the appropriate European Union regulatory authorities. The timeframe required for us to obtain such approvals is uncertain. In addition, we must pass a pre-approval inspection of our manufacturing facility by the FDA before KB103 can obtain marketing approval. In order to obtain approval, we will need to ensure that all of 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 KB103 is complex, novel and has not been validated for commercial use. In order to produce sufficient quantities of KB103 for future clinical trials and initial U.S. commercial demand, we will need to increase the scale of our manufacturing process. The production of KB103 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 KB103 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 intend to establish our own KB103 manufacturing facility, we expect 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.

Until such time as we establish our manufacturing facility that has been properly validated to comply with FDA cGMP requirements, we will not be able to independently manufacture material for our planned preclinical and clinical programs. Even following our establishment of a validated cGMP manufacturing facility, we intend to maintain third-party manufacturing capabilities in order to provide multiple sources of supply. In the event that the establishment of our own manufacturing facility is delayed and if these third-party manufacturers do not successfully carry out their contractual duties, meet expected deadlines or manufacture KB103 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 and the

clinical trials required for approval of KB103. 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 KB103 and would thereby have a material adverse effect on our business, financial condition, results of operations and prospects.

Building our own manufacturing facility will require additional investment, will be time consuming and may be subject to delays, including because of shortage of labor or compliance with regulatory requirements. In addition, building a manufacturing facility may cost more than we currently anticipate. Delays or problems in the build out of our manufacturing facility may adversely impact our ability to obtain regulatory approval and provide supply for the development and commercialization of KB103 as well as our financial condition.

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 KB103 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 KB103 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.

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

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

Since its enactment, there have been 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 ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. Further, in January 2017, Congress adopted a budget resolution for fiscal year 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of the PPACA. Following the passage of the Budget Resolution, in March 2017, the U.S. House of Representatives introduced legislation known as the American Health Care Act, which, if enacted, would amend or repeal significant portions of the PPACA. Among other changes, the American Health Care Act would repeal the annual fee on certain brand prescription drugs and biologics imposed on manufacturers and importers, eliminate penalties on individuals and employers that fail to maintain or provide minimum essential coverage, and create refundable tax credits to assist individuals in buying health insurance. The American Health Care Act would also make significant changes to Medicaid by, among other things, making Medicaid expansion optional for states, repealing the requirement that state Medicaid plans provide the same essential health benefits that are required by plans available on the exchanges, modifying federal funding, including implementing a per capita cap on federal payments to states, and changing certain eligibility requirements. While it is uncertain when or if the provisions in the American Health Care Act will become law, or the extent to which any changes may impact our business, it is clear that concrete steps are being taken to repeal and replace certain aspects of the PPACA.

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 2025 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 statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, there have been several recent U.S. 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.

Additional changes that may affect our business include 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 KB103 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 KB103 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 Health Care Program Anti-Kickback Statute, the federal civil and criminal laws and 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. The laws that will affect our operations include, but are not limited to:

- the federal Health Care Program 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. A person or entity no longer needs 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 and recent government cases against pharmaceutical and medical device manufacturers support the view that Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996, or 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, or 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 Centers for Medicare & Medicaid Services, or 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.

In addition, we may incur substantial costs in order 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.

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 we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. Our third-party manufacturing facility, as well as substantially all of our current supply of KB103 is located in Pittsburgh, Pennsylvania, and we do not have any existing back-up facilities in place or plans for such back-up facilities. 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 patent protection for our lead product candidate, KB103, any future product candidates we may develop and 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 technology similar or identical to ours, and our ability to successfully commercialize our current product candidate, any future product candidates we may develop and our technology may be adversely affected.

We do not currently own any patents. 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 both KB103 and future innovations related to our STAR-D platform. The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. We are actively prosecuting a patent application in front of the U.S. Patent and Trademark Office, or USPTO, directed, in part, to pharmaceutical formulations and methods of treating dystrophic epidermolysis bullosa using our KB103 product. A corresponding international application has also been filed in accordance with the Paris Cooperation treaty. In addition, we are seeking patent protection for key aspects of our viral platform technologies through a second patent application on file at the USPTO. We do not, however, yet know the outcome of these patent applications.

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. 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.

In addition, we may not be aware of all third-party intellectual property rights potentially relating to technologies similar to KB103. Publications of discoveries in the scientific literature often lag behind 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 product candidates 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 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 or license 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 use our proprietary technologies 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. Some of these patent applications have already been allowed or issued, while others may issue in the future. Since the areas

of gene delivery and gene therapeutics are competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed, and additional patents granted, in the future, as well as additional gene therapy research and development programs. Furthermore, 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 KB103 or related technologies, including, for example, interference proceedings, post grant review challenges, and inter partes review before the USPTO. 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 licensed patent portfolio may therefore have no deterrent effect.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their 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 KB103. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high, one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. In such a hypothetical situation, there is no assurance that a court of competent jurisdiction would find that KB103 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 KB103. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing KB103, or force us to cease some or all of 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 patents, should such patents issue, or we may be required to defend against claims of infringement. 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 condensedcondensed 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 our employees or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including potential competitors. Although we try 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 any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or 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.

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

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

Moreover, the patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain given the ever evolving and constantly shifting nature of precedential patent cases decided by both the U.S. Court of Appeals for the Federal Circuit and the U.S. Supreme Court. For instance, two cases involving diagnostic method claims and "gene patents" have recently been decided by the Supreme Court. On March 20, 2012, the Supreme Court issued a decision in Mayo Collaborative Services v. Prometheus Laboratories, Inc., or Prometheus, a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the Supreme Court, the addition of well-understood, routine or conventional activity such as "administering" or "determining" steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent-eligible subject matter. On July 3, 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied (and thus, the claim amounts to significantly more than the natural principle itself) should be rejected as directed to patent-ineligible subject matter. On June 13, 2013, the Supreme Court issued its decision in Association for Molecular Pathology v. Myriad Genetics, Inc., or Myriad, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. In its decision, the US Supreme Court held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 or BRCA2 genes, is not patent eligible subject matter; however, complementary DNA may be patent eligible.

Although the Supreme Court held in Myriad that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that potential activities that we undertake in the future may infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any situation involving third-party intellectual property rights, such as those directed to gene-related patent claims, if we are unsuccessful in defending against claims of patent infringement (*e.g.,* by asserting invalidity of the infringed patent in view of the Supreme Court's Myriad decision), we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects.

Moreover, we cannot assure you that our efforts to seek patent protection for our technology and product candidates will not be negatively impacted by the decisions described above, rulings in other cases, or changes in guidance or procedures issued by the USPTO. These decisions, the guidance issued by the USPTO (or changes thereto), and rulings in other cases could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property rights in the future.

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

We are currently in the process of registering our trademarks and trade names. Once registered, our trademarks or trade names 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 we need to build 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. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources 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 the 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 develop the specific technologies covered by the issued patents or pending patent applications 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 future issued claims, thus not infringing our intellectual property rights;
- others may circumvent our regulatory exclusivities, such as by pursuing approval of a competitive product candidate via the traditional approval pathway based on their own clinical data, rather than relying on the abbreviated pathway provided for biosimilar applicants;
- it is possible that our filed or future patent applications will not lead to issued patents;
- issued patents to which we 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 for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property

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

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 maintain the ability to substantially influence all matters submitted to stockholders for approval.

As of September 30, 2017, 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 38.9% 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 of 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.

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

Sales of a substantial number of shares of our common stock in the public market could occur at any time, subject to certain restrictions described below. These sales, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of September 30, 2017, we had outstanding 10,237,247 shares of common stock, of which 4,354,000 shares may be resold in the public market immediately without restriction, other than shares owned by our affiliates, which may be sold pursuant to Rule 144. However, the resale of an aggregate of 5,883,247 shares will be restricted as a result of lock-up agreements executed in connection with our initial public offering. We have registered all shares of common stock that we may issue under our equity compensation plans on a Registration Statement on Form S-8. These shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will rely, in part, on the research and reports that industry or financial analysts publish about us or our business. If securities analysts do not commence coverage of us, the trading price of our stock could decrease. Additionally, if one or more of the 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 is likely to be volatile. The stock market in general and the market for biopharmaceutical or pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular 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 have broad discretion in the use of our cash, including the net proceeds from our initial public offering, and may not use them effectively.

Our management will have broad discretion in the application of our cash, including the net proceeds from our initial public offering, and could spend the proceeds 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 KB103, KB104 and any other product candidates we may develop. Pending their use, we may invest our cash, including the net proceeds from our initial public offering, in a manner that does not produce income or that loses value.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or KB103.

We may seek additional capital through a combination of public and private equity offerings, debt financings, collaborations and licensing arrangements. To the extent that we raise additional capital through the sale of equity or debt securities, your ownership interest will be diluted and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or KB103, or grant licenses on terms unfavorable to us.

We are an "emerging growth company" and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and 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 Condensed 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 incur increased costs as a result of operating as a smaller reporting public company, and our management will be required to devote substantial time to new compliance initiatives.

As a smaller reporting public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act and rules subsequently implemented by the SEC and NASDAQ have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our Condensed financial statements.

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.

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 of 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.

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 Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Chief Executive Officer and Chief Financial Officer Pursuant to 18 U.S.C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

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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 13, 2017

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

By: /s/ Antony A. Riley
Antony Riley
Chief Financial Officer
(Principal Accounting and Financial 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 13, 2017

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

Exhibit 31.2

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

I, Antony A. Riley, 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 13, 2017

By: /s/ Antony A. Riley
Antony A. Riley
Chief Financial Officer
(Principal Accounting and Financial Officer)

Exhibit 32.1

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

I, 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 quarter ended September 30, 2017, (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 13, 2017

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

I, Antony A. Riley, Chief Financial 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 quarter ended September 30, 2017, (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 13, 2017

By: /s/ Antony A. Riley
Antony A. Riley
Chief Financial Officer
(Principal Accounting and Financial Officer)