As submitted confidentially to the Securities and Exchange Commission on July 17, 2017.
This draft registration statement has not been publicly filed with the Securities and Exchange Commission and all information herein remains strictly confidential.
Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
under
The Securities Act of 1933

KRYSTAL BIOTECH, INC.
(Exact name of registrant as specified in its charter)

Delaware	2836	81-0930882
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

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

Krish S. Krishnan
Chief Executive Officer
2100 Wharton Street, Suite 701
Pittsburgh, Pennsylvania 15203
(412) 586-5830
(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:

John W. Campbell III
Emiko Kurotsu
Katherine A. Shaia
Morrison & Foerster LLP
425 Market Street
San Francisco, California 94105
(415) 268-7000

Michael D. Maline
Seo Salimi
Goodwin Procter LLP
620 Eighth Avenue
New York, New York 10018
(212) 813-8800

Approximate date of commencement of proposed sale to the public: As soon as practicable after this registration statement becomes effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933 check the following box: ☐

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

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. (Check one):

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

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities to be Registered	Proposed Maximum Aggregate Offering Price(1)	Amount of Registration Fee(1)
Common Stock, par value $0.00001 per share	$	$

(1) Estimated solely for the purpose of computing the amount of registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended, based on an estimate of the proposed maximum aggregate offering price. Includes the offering price of additional shares of common stock that the underwriters have the option to purchase to cover over-allotments, if any.

The registrant hereby amends this registration statement on such date or dates as may be necessary to delay its effective date until the registrant shall file a further amendment which specifically states that this registration statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the registration statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

Table of Contents

The information in this preliminary prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities and we are not soliciting offers to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion. Dated , 2017.

Preliminary Prospectus

Shares

COMMON STOCK

We are offering shares of our common stock. This is our initial public offering and no public market currently exists for our shares. We anticipate that the initial public offering price of our common stock will be between $ and $ per share.

We intend to apply to list our common stock on the NASDAQ Capital Market under the symbol "KRYS."

We are an "emerging growth company" under the federal securities laws and are therefore subject to reduced public company reporting requirements.

Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

Investing in our common stock involves a high degree of risk. See "Risk Factors" beginning on page 10.

	Per Share	*Total*
Initial public offering price	$	$
Underwriting discounts and commissions(1)	$	$
Proceeds, before expenses, to Krystal Biotech, Inc.	$	$

(1) See "Underwriting" for additional information regarding underwriting compensation.

We have granted the underwriters the right to purchase up to an additional shares of common stock to cover over-allotments, if any.

The underwriters expect to deliver the shares of common stock to purchasers on , 2017.

Ladenburg Thalmann

, 2017

TABLE OF CONTENTS

You should rely only on the information contained in this prospectus or contained in any free writing prospectus prepared by or on behalf of us. Neither we nor the underwriters have authorized anyone to provide you with information different from, or in addition to, that contained in this prospectus or any related free writing prospectus. This prospectus is an offer to sell only the shares offered hereby but only under circumstances and in jurisdictions where it is lawful to do so. The information contained in this prospectus is current only as of its date, regardless of its delivery. Our business, financial condition, results of operations and prospects may have changed since that date.

Through and including , 2017 (25 days after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to their unsold allotments or subscriptions.

For investors outside the United States: neither we nor any of the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus.

PROSPECTUS SUMMARY

This summary highlights information contained elsewhere in this prospectus. It may not contain all the information that may be important to you. You should read the entire prospectus carefully, including the section entitled "Risk Factors" and our financial statements and the related notes included elsewhere in this prospectus before making an investment decision to purchase shares of our common stock.

In this prospectus, unless we indicate otherwise or the context requires, references to the "Company," "Krystal," "we," "our," "ours," and "us" refer to Krystal Biotech, Inc. The following summary is qualified in its entirety by the more detailed information and financial statements and notes thereto included elsewhere in this prospectus.

Our Business

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 a patent pending engineered 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 the 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 to expand our pipeline to include other dermatological indications in the future.

Our lead product candidate, KB103, is currently in preclinical development 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. KB103 is a replication-defective, non-integrating viral vector that has been engineered employing our STAR-D platform to deliver functional human COL7A1 genes directly to the patients' dividing and non-dividing skin cells. Preclinical studies evaluating intradermal and topical delivery of KB103 have been completed, and have demonstrated successful introduction of a functional COL7A1 gene to the host cells and subsequent expression of COL7. We intend to file an Investigational New Drug, or IND, application for KB103 with the U.S. Food and Drug Administration, or FDA, in January 2018.

We have commenced preclinical studies on our second pipeline compound, KB104, to treat Netherton Syndrome, a severe autosomal recessive form of ichthyosis characterized by chronic skin inflammation, itchiness, dehydration and stunted growth. We intend to file an IND on KB104 in the third quarter of 2018 and begin clinical studies in the first quarter of 2019. We have also commenced research activities on ichthyosis vulgaris, and intend to start research activities on treatments for psoriasis, atopic dermatitis and chronic wounds in the fourth quarter of 2017. Following successful completion of a Phase 1 clinical trial of KB103, we intend to design, build and validate a commercial-scale current good manufacturing practices, or cGMP, facility for the upstream and downstream manufacturing processes of products based on our STAR-D platform.

Our management team includes individuals with expertise in gene therapy, product development, and manufacturing and commercialization in the biotechnology industry. Our scientific team collectively has over 20 years of experience in herpes simplex virus, or HSV, engineering and purification, providing the expertise needed to successfully optimize our HSV-1 vector production process. In addition, we are guided by key opinion leaders, or KOLs, who are generally accepted in the medical and scientific communities to be leading experts in the DEB and orphan dermatological disease space. Our KOLs include Dr. Peter

Marinkovich of the Department of Dermatology of Stanford University and Dr. Andrew South of the Department of Dermatology of Thomas Jefferson University.

Our Strengths

We believe we are the first biotechnology company to seek to use gene therapy to develop products for dermatological indications that can be used without requiring individually customized treatment, which we refer to as an "off-the-shelf" treatment. We believe our organization and technology benefit from a singular set of strengths that will allow us to create and establish a leadership position in developing gene therapy treatments for dermatological indications. These strengths include:

- A first mover advantage in dermatological gene therapy with regards to:
  - An off-the-shelf gene therapy product candidate, and
  - Topical gene therapy application;
- Our proprietary, integrated STAR-D gene therapy platform;
- The significant affinity to the skin and high payload capacity of our HSV-1 viral vector, which will allow us to deliver single and multiple genes to treat orphan and other dermatological indications;
- A proprietary process for both upstream (vector production) and downstream (purification) portions of the manufacturing process, which positions us to maximize scalability, quality and reliability;
- A scientific team with expertise in the HSV-1 viral vector; and
- A management team with a track record in developing drugs from research to approval.

Our STAR-D Gene Therapy Platform

We believe our STAR-D platform is an optimal approach to treating dermatological conditions due to the nature of the HSV-1 viral vector we have created. We believe that certain inherent features of the HSV-1 virus, combined with our ability to strategically modify the virus in the form we employ as our gene delivery backbone, provides our STAR-D platform with several advantages over other viral vector platforms for use in dermatological applications. These characteristics of our viral vector include the following:

- Non-integrating nature
- Large payload capacity
- Skin tropism resulting in high transduction efficiencies
- Low immunogenicity
- Stability
- Reproducible manufacturing and scalability
- Existing regulatory precedent

Our Lead Product Candidate: KB-103 for the Treatment of DEB

Our lead product candidate, KB103, is currently in preclinical development. KB103 seeks to use gene therapy to treat 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 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. We estimate that there are presently 3,200 to 3,500 diagnosed DEB patients in the world, with the majority of such patients in the European Union and United States, and lesser populations in Japan and Canada.

We believe our approach to treating DEB with KB103 is novel. The current standard of care for DEB patients is limited to palliative measures which seek to provide relief from some of the symptoms of DEB but do not meaningfully impact disease outcomes. There is no approved treatment for DEB, and the current therapeutic DEB treatments in clinical development of which we are aware are limited to two companies and two universities employing autologous approaches. Autologous treatments use a patient’s own tissues and cells to manufacture an individualized therapy. Such therapies are expensive, invasive and time consuming to use, and require highly sophisticated medical teams and procedures. In contrast, KB103 is designed to be an off-the-shelf treatment for DEB that can be applied either intra-dermally or topically to a patient’s skin, every three to four months. Unlike the current standard of care, KB103 seeks to treat DEB at the molecular level through gene therapy, and is intended to be a non-invasive treatment that can be used without requiring hospitalization or individual customization.

In October 2016, we had a pre-IND meeting with the FDA. Based on responses from the FDA, we plan to submit an IND to initiate a Phase 1/2 clinical trial of KB103 in the first quarter of 2018. Results of this trial, which we expect to receive in mid-2018, will guide us in finalizing the design of a pivotal Phase 3 clinical trial. If successful, we believe the results of this Phase 3 trial could support submission of a Biologics License Application, or BLA, to the FDA in the United States and a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, in Europe for our KB103 product candidate for the treatment of DEB. 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.

Risks Associated with our Business

Our business is subject to numerous risks and uncertainties, including those highlighted in the section entitled “Risk Factors” immediately following this prospectus summary. These risks include, but are not limited to, the following:

- We have never generated revenue from product sales and may never be profitable.
- Our independent registered public accounting firm has expressed substantial doubt about our ability to continue as a going concern.
- We have a limited operating history and are very early in our development efforts.
- Our lead product candidate, KB103, is still in preclinical development.
- We may be unable to advance any of our product candidates to clinical trials, obtain regulatory approval and ultimately commercialize our product candidates.
- We have not tested any of our product candidates in clinical trials, and success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials.
- 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.

- 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.
- 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.
- Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our KB103 gene therapy product candidate and adversely affect our ability to conduct our business or obtain regulatory approvals for KB103.
- We may be unable to obtain orphan drug exclusivity for KB103 or any other 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.
- 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.
- Even if we obtain and maintain approval for KB103 from the FDA, we may never obtain approval for KB103 outside of the United States, which would limit our market opportunities and adversely affect our business. 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 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.
- We have a limited number of employees and limited corporate infrastructure, and may experience difficulties in managing growth.
- The commercial success of KB103 will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community.
- 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.

Corporate Information

Our principal executive offices are located at 2100 Wharton Street, Suite 701, Pittsburgh, Pennsylvania 15203, and our telephone number is (412) 586-5830. Our website is www.krystalbio.com. Information contained on, or that can be accessed through, our website is not incorporated by reference into this prospectus, and you should not consider information on our website to be part of this prospectus. We formed as a limited liability company in California under the name Krystal Biotech, LLC in December 2015 and commenced business in April 2016. We converted to a Delaware corporation in March 2017.

Implications of Being an Emerging Growth Company

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. An emerging growth company may take advantage of relief from certain

reporting requirements and other burdens that are otherwise applicable generally to public companies. These provisions include:

- reduced obligations with respect to financial data, including presenting only two years of audited financial statements and only two years of selected financial data in this prospectus;
- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act;
- reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements; and
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements.

We may take advantage of these provisions for up to five years or such earlier time that we no longer qualify as an emerging growth company. We would cease to be an emerging growth company if we have more than $1.07 billion in annual revenue, have more than $700 million in market value of our capital stock held by non-affiliates or issue more than $1.0 billion of non-convertible debt over a three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens. For example, we intend to take advantage of the reduced reporting requirements with respect to disclosure regarding our executive compensation arrangements, have presented only two years of audited financial statements and only two years of related “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure in this prospectus, and have taken advantage of the exemption from auditor attestation on the effectiveness of our internal control over financial reporting. To the extent that we take advantage of these reduced reporting burdens, the information that we provide stockholders may be different than you might obtain from other public companies in which you hold equity interests.

In addition, under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards 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, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

THE OFFERING

Common stock to be offered by us	shares
Common stock to be outstanding immediately following this offering	shares
Option to purchase additional shares	We have granted to the underwriters the option, exercisable for 30 days from the date of this prospectus, to purchase up to additional shares of our common stock.
Use of proceeds	We estimate that the net proceeds from the sale of our common stock sold in this offering will be approximately $ million, based on an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, after deducting the underwriting discounts and commissions and estimated offering expenses. We intend to use the net proceeds from this offering to fund the preclinical and clinical development of KB103 for the treatment of DEB and for the preclinical development of KB104 for the treatment of Netherton Syndrome, to fund research for the development of treatments for additional dermatological indications, to design and build our in-house manufacturing facility, and for general corporate purposes, including working capital. See the section entitled “Use of Proceeds.”
Risk factors	You should read the section entitled “Risk Factors” and other information included in this prospectus for a discussion of factors you should consider carefully before deciding to invest in shares of our common stock.
Proposed NASDAQ Capital Market symbol	“KRYS”

The number of shares of our common stock to be outstanding following this offering is based on shares of our common stock outstanding as of March 31, 2017. It excludes:

- 25,263 shares of our common stock issuable upon the exercise of options outstanding as of March 31, 2017, with an exercise price of $11.07 per share;
- 10,211 shares of our common stock issuable upon the exercise of options granted after March 31, 2017 through June 30, 2017, with a weighted average exercise price of $22.00 per share; and
- 7,426 shares of common stock reserved for future issuance under our Krystal Biotech, Inc. 2017 Stock Incentive Plan as of June 30, 2017, and any future increase in shares reserved for issuance under such plan.

Unless otherwise noted, the information in this prospectus reflects and assumes the following:

- The conversion of all of our outstanding common units and preferred units issued during the period we operated as a limited liability company into shares of common stock and preferred stock on a one-to-one basis, and the conversion of all issued options to purchase incentive units issued during such period into options to purchase an identical number of shares of common stock at the same exercise price per share;

- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 39,914 shares of our common stock effective immediately prior to the completion of this offering;
- the conversion of all of our outstanding convertible notes and accrued interest into an aggregate of shares of our common stock, effective immediately prior to the completion of this offering;
- a 1-to- forward stock split, which will occur immediately prior to the completion of this offering;
- the filing and effectiveness of our amended and restated certificate of incorporation in Delaware and the adoption of our amended and restated bylaws, both of which will occur immediately prior to the completion of this offering;
- no exercise of outstanding options; and
- no exercise of the underwriters' option to purchase additional shares.

We operated as a California limited liability company from inception of operations on April 15, 2016 until our conversion into a Delaware corporation on March 31, 2017 and were managed by a board of managers prior to conversion. References herein to actions taken by our board of directors include actions taken by our board of managers prior to conversion. References herein to "stock," "common stock," "preferred stock," and "options to purchase common stock" during periods prior to conversion are to "units," "common units," "preferred units" and "options to purchase incentive units," respectively.

SUMMARY SELECTED FINANCIAL DATA

The following summary financial data for the year ended December 31, 2016 has been derived from our audited financial statements appearing elsewhere in this prospectus. The summary financial data as of March 31, 2017 and for the three months ended March 31, 2016 and 2017 have been derived from our unaudited financial statements included elsewhere in this prospectus. The unaudited financial statements have been prepared on a basis consistent with our audited financial statements and, in the opinion of management, contain all adjustments, consisting only of normal and recurring adjustments, necessary for a fair statement of such financial data. You should read this data together with our audited financial statements and related notes appearing elsewhere in this prospectus and the information under the captions "Risk Factors," "Capitalization," "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results are not necessarily indicative of our future results, and our operating results for the three-month period ended March 31, 2017 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2017 or any other interim periods or any future year or period.

(in thousands, except shares, units and per share)	Year Ended December 31, 2016	Three Months Ended March 31, 2016	Three Months Ended March 31, 2017
		(unaudited)	
Statements of operations data:			
Revenues			
Revenues	$ —	$ —	$ —
Total revenues	—	—	—
Expenses			
Research and development	741	—	319
General and administrative	402	—	146
Total operating expenses	1,143	—	465
Loss from operations	(1,143)	—	(465)
Other Income (Expense)			
Interest expense	(7)	—	(29)
Total other income (expense)	(7)	—	(29)
Net loss	$ (1,150)	—	$ (494)
Net loss applicable to stockholders and members	$ (1,150)	—	$ (494)
Net loss attributable to common stockholders per share[(1)]:			
Basic and diluted	$ (5.89)	—	$ (0.64)
Basic and diluted, pro forma (unaudited)	$ (5.51)	—	$ (0.50)
Weighted-average common shares and common units outstanding			
Basic and diluted	194,998	—	775,752
Basic and diluted, pro forma (unaudited)	—	—	930,826

(1) See Note 2 to our financial statements included elsewhere in this prospectus for a description of the method used to calculate the basic and diluted net loss per share and pro forma basic and diluted net loss per share.

(in thousands)	As of March 31, 2017		
	Actual	Pro Forma(1)	Pro Forma As Adjusted(2)(3)
		(unaudited)	
Balance sheet data:			
Cash	$ 1,959	$	$
Working capital	2,047		
Total assets	2,151		
Accrued expenses	27		
Related party promissory notes	698		
Total liabilities	2,265		
Convertible preferred stock	1,406		
Total stockholders' (deficit) equity	(1,520)		

(1) Pro forma reflects the automatic conversion of all outstanding shares of our preferred stock on March 31, 2017 into 39,914 shares of our common stock immediately prior to the completion of this offering and the conversion of $ in convertible notes and accrued interest outstanding into shares of our common stock.

(2) Pro forma as adjusted reflects the sale of shares of our common stock offered in this offering, assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

(3) A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and total stockholders' (deficit) equity by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. A share increase in the number of shares offered by us together with a $1.00 increase in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase each of cash and total stockholders' (deficit) equity by approximately $ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us. Conversely, a share decrease in the number of shares offered by us together with a $1.00 decrease in the assumed initial public offering price of $ per share, the midpoint of the initial public offering price range set forth on the cover of this prospectus, would decrease each of cash and total stockholders' (deficit) equity by approximately $ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should carefully consider the following risks and all other information contained in this prospectus, including our financial statements and the related notes, before investing in our common stock. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, also may become important factors that affect us. If any of the following risks occur, our business, financial condition, results of operations and prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL CAPITAL

We have never generated revenue and may never be profitable.

We have not generated any revenue to date and 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.

Our independent registered public accounting firm has expressed substantial doubt about our ability to continue as a going concern.

As described in their audit report, our auditors have included an explanatory paragraph that states that we have incurred recurring losses and negative cash flows from operations since inception and have an accumulated deficit at December 31, 2016 of $1.2 million. These matters raise substantial doubt about our ability to continue as a going concern. Our consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. If we cannot continue as a viable entity, our securityholders may lose some or all of their investment in us.

Even if this offering is successful, 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.

We estimate that the net proceeds from this offering will be approximately $ million, based on an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. 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 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 two gene therapy products, uniQure N.V.'s Glybera® and GlaxoSmithKline's Strimvelis™, have received marketing authorization from the European Commission, and no gene therapy product has received marketing authorization by the FDA. 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 no 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 six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Even 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 for KB103 or other product candidates in the United States 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 interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing, or distribution activities.

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

We may be subject to claims asserting that 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 THIS OFFERING AND OWNERSHIP OF OUR COMMON STOCK

After this offering, our Chief Executive Officer and Chairman of the Board of Directors and our founder, Chief Operating Officer and director will maintain the ability to control all matters submitted to stockholders for approval.

Upon completion of this offering, assuming the sale by us of all of the shares set forth on the cover page of this prospectus (other than shares subject to the underwriter's option to purchase additional shares) 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, will, in the aggregate, beneficially own shares representing approximately % of our capital stock. As a result, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons would control 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 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. After this offering, we will have outstanding shares of common stock based on the number of shares outstanding as of , 2017, all of which 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 shares will be restricted as a result of lock-up agreements executed in conjunction with this offering, as described in the "Shares Eligible for Future Sale" and "Underwriting" sections of this prospectus. We will register 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 described in the "Shares Eligible for Future Sale" section of this prospectus.

If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.

The public offering price of our common stock will be substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. To the extent outstanding options are exercised, you will incur further dilution. Based on the assumed public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, you will experience immediate dilution of $ per share, representing the difference between the assumed public offering price and our as adjusted net tangible book value per share as of after giving effect to this offering. See "Dilution."

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 purchasers of our common stock in this offering.

The offering price for the shares of our common stock sold in this offering will be determined by negotiation between the representatives of the underwriters and us. This price may not reflect the market price of our common stock following this offering. In addition, the market price of our common stock is likely to be highly volatile due to 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.

An active trading market for our common stock may not develop and you may not be able to resell your shares at or above the initial public offering price.

Prior to this offering, there has been no public market for shares of our common stock. Although we have applied to list our common stock on the NASDAQ Capital Market, an active trading market for our shares may never develop or be sustained following this offering. The initial public offering price of our common stock will be determined through negotiations between us and the underwriters. This initial public offering price may not be indicative of the market price of our common stock after this offering. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the initial public offering price or at the time that they would like to sell.

We have broad discretion in the use of our cash, including the net proceeds from this offering, and may not use them effectively.

Our management will have broad discretion in the application of our cash, including the net proceeds from this 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 this offering, in a manner that does not produce income or that loses value. See “Use of Proceeds.”

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 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.

We have taken advantage of reduced reporting burdens in this prospectus. In particular, we have not included all of the executive compensation information that would be required if we were not an emerging growth company. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be 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 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 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or 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.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. The forward-looking statements are contained principally in the sections entitled “Prospectus Summary,” “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and “Business.” Forward-looking statements include information concerning our strategy, future operations, future financial position, future revenue, projected expenses, prospects and plans and objectives of management. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “target,” “will,” “would” or similar expressions and the negatives of those terms.

Forward-looking statements contained in this prospectus include, but are not limited to, statements about the following:

- the initiation, timing, progress and results of preclinical and clinical trials for KB103 and any other product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs;
- the timing, scope or results of regulatory filings and approvals, including timing of final FDA marketing and other regulatory approval of KB103;
- our ability to achieve certain accelerated or orphan drug designations from the FDA;
- our estimates regarding the potential market opportunity for KB103;
- our research and development programs for our product candidates;
- our plans and ability to successfully develop and commercialize our product candidates, including KB103 and KB104;
- our ability to identify and develop new product candidates;
- our ability to identify, recruit and retain key personnel;
- our commercialization, marketing and manufacturing capabilities and strategy;
- the implementation of our business model, strategic plans for our business, product candidates and technology;
- the scalability and commercial viability of our proprietary manufacturing methods and processes;
- the rate and degree of market acceptance and clinical utility of our product candidates and gene therapy, in general;
- our competitive position;
- our intellectual property position and our ability to protect and enforce our intellectual property;
- our financial performance;
- developments and projections relating to our competitors and our industry;
- our ability to establish and maintain collaborations or obtain additional funding;
- our expectations related to the use of proceeds from this offering;
- our estimates regarding expenses, future revenue, capital requirements and needs for or ability to obtain additional financing;
- the impact of laws and regulations; and
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act.

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

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

INDUSTRY AND MARKET DATA

We obtained the industry, statistical and market data in this prospectus from our own internal estimates and research, as well as from industry and general publications and research, surveys and studies conducted by third parties. In presenting this information, we have made assumptions based on such data and other similar sources, and on our knowledge of, and our experience to date in, the potential markets for our product candidates. Although we believe the data from these third-party sources is reliable, we have not independently verified any third-party information. The industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the section entitled “Risk Factors.” These and other factors could cause results to differ materially from those expressed in the estimates made by third parties and by us.

USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of shares of our common stock in this offering will be approximately $ million (or $ million if the underwriters exercise in full their option to purchase additional shares), assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the net proceeds to us from this offering by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each 1.0 million increase (decrease) in the number of shares offered by us, as set forth on the cover of this prospectus, would increase (decrease) the net proceeds to us by $ million, assuming the assumed initial public offering price per share remains the same, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

As of March 31, 2017, we had cash of approximately $2.0 million. We intend to use the net proceeds from this offering, together with our existing cash, as follows:

- Approximately $ to fund the development of KB103 through the first half of 2018, including preclinical development and the submission of an IND and potential completion of our currently planned Phase 1/2 study of KB103;
- Approximately $ to advance the development of KB104, our second pipeline compound being developed to treat Netherton Syndrome, through the potential submission of an IND during the second half of 2018;
- Approximately $ to fund research activities through the end of 2018 on the application of our STAR-D platform to the development of treatments for additional dermatological indications such as ichthyosis vulgaris, psoriasis, atopic dermatitis and chronic wounds;
- Approximately $ to design and build a current good manufacturing practices, or cGMP, certified manufacturing facility for scale-up production of our pipeline compounds; and
- The balance for general corporate purposes, including general and administrative expenses and working capital.

We believe that our current cash, along with the net proceeds from this offering, will be sufficient for us to fund our operating expenses and capital expenditure requirements for the next months .

The expected net proceeds of this offering will not be sufficient for us to fund any of our product candidates, including KB103, through regulatory approval, and we will need to raise substantial additional capital to complete the development and commercialization of our product candidates, as well as to establish an in-house manufacturing facility.

The amounts and timing of our actual expenditures will depend on numerous factors, including the progress of our preclinical and clinical trials and other development and commercialization efforts for KB103 and our other product candidates, as well as the amount of cash used in our operations. Although we have no present intention or commitment to do so, we may use a portion of the net proceeds for the acquisition of, or investment in, technologies, intellectual property or businesses that complement our business.

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with complete certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the actual amounts that we will spend on the uses set forth above. We may find it necessary or advisable

to use the net proceeds for other purposes, and our management will retain broad discretion over the allocation of the net proceeds of this offering. Pending the uses described above, we plan to invest the net proceeds from this offering in short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

DIVIDEND POLICY

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings for use in the operation of our business and do not anticipate paying any dividends on our common stock in the foreseeable future. Any future determination to declare dividends will be made at the discretion of our board of directors and will depend on our financial condition, operating results, capital requirements, general business conditions and other factors that our board of directors may deem relevant.

CAPITALIZATION

The following table sets forth our cash and our capitalization as of March 31, 2017:

- on an actual basis;
- on a pro forma basis to give effect to: (i) the automatic conversion of all outstanding shares of our preferred stock into an aggregate 39,914 shares of common stock, which conversion will occur immediately prior to the closing of this offering; (ii) the conversion all outstanding convertible notes and accrued interest into an aggregate of shares of common stock effective immediately prior to the closing of this offering; and (iii) the 1-to- forward stock split, which will occur immediately prior to the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of shares of common stock in this offering at an assumed public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us and excluding any additional shares of common stock that may be issuable upon the exercise of the underwriters' option to purchase additional shares.

The pro forma as adjusted information set forth in the table below is illustrative only and will be adjusted based on the actual initial public offering price and other final terms of this offering. You should read this information together with our financial statements and the related notes thereto and the information set forth under the headings "Selected Financial Information" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" appearing elsewhere in this prospectus.

	As of March 31, 2017		
(in thousands, except share and per share amounts)	**Actual**	**Pro Forma**	**Pro Forma As Adjusted(1)**
		(unaudited)	
Cash	$ 1,959	$	$
Convertible promissory notes			
Related party convertible promissory notes	698		
Convertible promissory notes	1,445		
Total convertible promissory notes	2,143		
Convertible preferred stock			
Convertible preferred stock, $0.00001 par value; 100,000 shares authorized, 39,914 shares issued and outstanding at March 31, 2017, no shares authorized, issued and outstanding pro forma or pro forma as adjusted (unaudited)	1,406		
Total convertible preferred stock	1,406		
Stockholders' equity:			
Common stock; $0.00001 par value; 10,000,000 shares authorized, 775,752 shares issued and outstanding at March 31, 2017, and 10,000,000 shares authorized, shares issued and outstanding pro forma, and shares authorized, issued and outstanding pro forma as adjusted (unaudited)	—		
Additional paid-in capital	124		
Accumulated deficit	(1,644)		
Total stockholders' (deficit) equity	(1,520)		
Total capitalization	$ 2,029	$	$

(1) A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) each of cash, additional paid-in capital, total stockholders' (deficit) equity and total capitalization by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1,000,000 in the number of shares we are offering would increase (decrease) cash, additional paid-in capital, total stockholders' (deficit) equity and total capitalization by approximately $ million, assuming the assumed initial public offering price remains the same, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The table above excludes the following:

- 25,263 shares of our common stock issuable upon the exercise of options outstanding as of March 31, 2017, with an exercise price of $11.07 per share;
- 10,211 shares of our common stock issuable upon the exercise of options granted after March 31, 2017 through June 30, 2017, with a weighted average exercise price of $22.00 per share; and
- 7,426 shares of common stock reserved for future issuance under our Krystal Biotech, Inc. 2017 Stock Incentive Plan as of June 30, 2017, and any future increase in shares reserved for issuance under such plan.

DILUTION

If you invest in our common stock, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering. Net tangible book value dilution per share represents the difference between the amount per share paid by purchasers of shares of common stock in this offering and the pro forma as adjusted net tangible book value per share of common stock immediately after the completion of this offering.

As of March 31, 2017, our net tangible book value was approximately $(114) thousand, or $(0.15) per share of common stock. Net tangible book value per share represents the amount of our tangible assets less our liabilities divided by the total number of shares of our common stock outstanding as of March 31, 2017. Our net tangible book value calculation excludes intangible assets and deferred tax liability associated with intangible assets. Our pro forma net tangible book value as of March 31, 2017 was $ million, or $ per share, based on the total number of shares of our common stock outstanding as of March 31, 2017, after giving effect to: (i) the automatic conversion of all outstanding shares of our preferred stock as of March 31, 2017 into an aggregate of 39,914 shares of common stock, which conversion will occur immediately prior to the closing of this offering; (ii) the conversion of all outstanding convertible notes and accrued interest into an aggregate of shares of common stock effective immediately prior to the closing of this offering; and (iii) the 1-to- forward stock split, which will occur immediately prior to the closing of this offering.

After giving effect to the sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of March 31, 2017 would have been $ million, or $ per share. This represents an immediate increase in pro forma as adjusted net tangible book value of $ per share to our existing stockholders and an immediate dilution of $ per share to new investors participating in this offering.

The following table illustrates this dilution on a per share basis:

Assumed initial public offering price per share		$
Historical net tangible book value per share as of March 31, 2017	$ (0.15)	
Pro forma decrease per share attributable to the pro forma transactions and other adjustments described above		
Pro forma net tangible book value per share as of March 31, 2017	$	
Increase in pro forma net tangible book value per share attributable to new investors in this offering		
Pro forma as adjusted net tangible book value per share immediately after this offering		
Dilution in pro forma net tangible book value per share to new investors in this offering		$

Each $1.00 increase (decrease) in the assumed initial public offering price of $, the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) our pro forma as adjusted net tangible book value per share after this offering by $ per share and the dilution per share to new investors participating in this offering by $ per share, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each 1.0 million increase (decrease) in the number of shares offered by us would increase (decrease) the pro forma as adjusted net tangible book value per share after this offering by $ per share and the dilution

per share to new investors participating in this offering by $ per share, assuming that the assumed initial public offering price remains the same, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise in full their option to purchase up to additional shares of common stock to cover over-allotments, if any, the pro forma as adjusted net tangible book value per share after giving effect to this offering would be $ per share, representing an immediate increase to existing stockholders of $ per share and immediate dilution to new investors participating in this offering of $ per share assuming that the assumed initial public offering price remains the same, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

To the extent any outstanding options to purchase common stock are exercised, new investors would experience further dilution.

The following table summarizes, on a pro forma basis as of March 31, 2017, the differences between the number of shares of common stock purchased from us, the total cash consideration and the average price per share paid to us by existing stockholders and by new investors purchasing shares in this offering, at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders		%	$	%	$
New public investors					$
Total		100%	$	100%	

If the underwriters exercise their option to purchase additional shares in full, the number of shares of common stock held by existing stockholders will be reduced to % of the total number of shares of common stock to be outstanding after this offering, and the number of shares of common stock held by investors participating in this offering will be further increased to % of the total number of shares of common stock to be outstanding after this offering.

The number of shares of our common stock to be outstanding after this offering excludes:

- 25,263 shares of our common stock issuable upon the exercise of options outstanding on March 31, 2017, with an exercise price of $11.07 per share;
- 10,211 shares of our common stock issuable upon the exercise of options granted after March 31, 2017 through June 30, 2017, with a weighted average exercise price of $22.00 per share; and
- 7,426 shares of common stock reserved for future issuance under our Krystal Biotech, Inc. 2017 Stock Incentive Plan as of June 30, 2017, and any future increase in shares reserved for issuance under such plan.

SELECTED FINANCIAL DATA

The selected statements of operation data for the year ended December 31, 2016 has been derived from our audited financial statements appearing elsewhere in this prospectus. The selected financial data as of March 31, 2017 and for the three months ended March 31, 2016 and 2017 have been derived from our unaudited financial statements included elsewhere in this prospectus. The unaudited financial statements have been prepared on a basis consistent with our audited financial statements and, in the opinion of our management, contain all adjustments, consisting only of normal and recurring adjustments, necessary for a fair statement of such financial data. You should read this data together with our audited financial statements and related notes appearing elsewhere in this prospectus and the information under the captions "Risk Factors," "Capitalization," and "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results are not necessarily indicative of our future results, and our operating results for the three-month period ended March 31, 2017 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2017 or any other interim periods or any future year or period.

(in thousands, except shares, units and per share data)	Year Ended December 31, 2016	Three Months Ended March 31, 2016	Three Months Ended March 31, 2017
		(unaudited)	
Statements of operations data:			
Revenues			
Revenues	$ —	$ —	$ —
Total revenues	—	—	—
Expenses			
Research and development	741	—	319
General and administrative	402	—	146
Total operating expenses	1,143	—	465
Loss from operations	(1,143)	—	(465)
Other Income (Expense)			
Interest expense	(7)	—	(29)
Total other income (expense)	(7)	—	(29)
Net loss	(1,150)	—	(494)
Net loss applicable to stockholders and members	$ (1,150)	—	$ (494)
Net loss attributable to common stockholders per share:			
Basic and diluted	$ (5.89)	—	$ (0.64)
Weighted-average common shares and common units outstanding			
Basic and diluted	194,998	—	775,752

(in thousands)	As of December 31, 2016	As of March 31, 2017 (unaudited)
Balance sheet data:		
Cash	$ 1,923	$ 1,959
Working capital	2,126	2,047
Total assets	2,182	2,151
Accrued expenses	1	27
Related party promissory notes	698	698
Total liabilities	1,893	2,265
Convertible preferred units and preferred stock	—	1,406
Total stockholders' and members' equity (deficit)	289	(1,520)

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the section entitled "Selected Financial Data" and our financial statements and related notes appearing in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

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. We estimate that there are 3,200 to 3,500 diagnosed DEB patients in the world presently, with the majority of such patients in the European Union and United States, and lesser populations in 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 (unaudited), 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. We have primarily financed our operations through the issuance of our equity securities and debt financings. At March 31, 2017 (unaudited), we had received $1.4 million in gross proceeds from the issuance of equity securities and $2.1 million in gross proceeds from debt financings. At March 31, 2017 (unaudited), our cash was approximately $2.0 million.

Since operations began, we have incurred operating losses. Our net losses were $1.2 million and $494 thousand for the year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited), respectively. At March 31, 2017 (unaudited), we had accumulated a deficit of $1.6 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 the net proceeds from this offering and from these other sources will be sufficient to fund our planned preclinical and clinical studies or our operations through this period. These 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

The results of operations contained within this Management's Discussion and Analysis do not contain comparative results to the year ended December 31, 2016 because we did not exist as a legal entity prior to December 20, 2015. Additionally, the comparison of financial results for the three months ended March 31, 2016 and 2017 (unaudited), present no financial activity in the first quarter of 2016 since we did not commence operations until April 15, 2016.

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 on our convertible promissory notes, which is partially 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 financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the amounts reported in the 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.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this prospectus, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued research and development expenses, current assets and other current liabilities. This process involves reviewing open contracts and commitments, communicating with our personnel to identify services that have been performed for us and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued research and development expenses, current assets and other current liabilities as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses, prepaid assets and other current liabilities include fees paid to contract manufacturers made in connection with the manufacturing of clinical trials materials.

We base our expenses related to clinical manufacturing on our estimates of the services performed pursuant to contracts with the entities producing clinical materials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under these types of contracts depend heavily upon the successful completion of many separate tasks involved in the manufacturing of drug product. In accruing service fees, we estimate the time period over which services will be performed, and the actual services performed in each period. If

our estimates of the status and timing of services performed differs from the actual status and timing of services performed we may report amounts that are too high or too low in any particular period. To date, there have been no material differences from our estimates to the amount actually incurred.

Stock-Based Compensation

We have applied the fair value recognition provisions of Financial Accounting Standards Board Accounting Standards Codification, or ASC, Topic 718, *Compensation—Stock Compensation*, or ASC 718, to account for stock-based compensation for employees and ASC 718 and ASC 505, *Equity*, or ASC 505, for non-employees. We recognize compensation costs related to stock options granted to employees based on the estimated fair value of the awards on the date of grant. Stock compensation related to non-employee awards is re-measured at each reporting period until the awards are vested. Described below is the methodology we have utilized in measuring stock-based compensation expense.

Determining the amount of stock-based compensation to be recorded requires us to develop estimates of the fair value of stock-based awards as of their measurement date. We recognize stock-based compensation expense over the requisite service period, which is the vesting period of the award. Calculating the fair value of stock-based awards requires that we make highly subjective assumptions. We use the Black-Scholes option pricing model to value our stock option awards. Use of this valuation methodology requires that we make assumptions as to the volatility of our common stock, the fair value of our common stock on the measurement date, the expected term of our stock options, the risk free interest rate for a period that approximates the expected term of our stock options and our expected dividend yield. Because we are a privately held company with a limited operating history, we utilize data from a representative group of publicly traded companies to estimate expected stock price volatility. We selected representative companies from the biopharmaceutical industry with characteristics similar to us. We use the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, *Share-Based Payment* as we do not have sufficient historical stock option activity data to provide a reasonable basis upon which to estimate the expected term of stock options granted to employees. For non-employee grants, we use an expected term equal to the remaining contractual term of the award. We utilize a dividend yield of zero based on the fact that we have never paid cash dividends and have no current intention of paying cash dividends. The risk-free interest rate used for each grant is based on the U.S. Treasury yield curve in effect at the time of grant for instruments with a similar expected life.

Under ASC 718, we are required to estimate the level of forfeitures expected to occur and record stock-based compensation expense only for those awards that we ultimately expect will vest. For all periods presented, our estimated annual forfeiture rate was 0%.

Stock-based compensation expense includes options granted to employees and non-employees and has been reported in our statements of operations and comprehensive loss as follows (in thousands):

	Year Ended December 31, 2016	Three Months Ended March 31, 2016	Three Months Ended March 31, 2017 (unaudited)
Research and development	$ 25	$—	$ 32
General and administrative	8	—	59
Total	$ 33	$—	$ 91

We estimated the fair value of stock options of each employee stock award at the grant date using assumptions regarding the fair value of the underlying common stock on each grant date and the following additional assumptions:

	Year Ended December 31, 2016
Employees	
Expected volatility	80%
Expected term of the award (in years)	6.25
Risk-free interest rate	1.97%
Expected dividend yield	—
Non-employees	
Expected volatility	80%
Expected time to maturity (in years)	6.25
Risk-free interest rate	1.97%
Expected dividend yield	—

At March 31, 2017, we had approximately $319 thousand of total unrecognized compensation expense, net of related forfeiture estimates, which we expect to recognize over a weighted-average remaining vesting period of approximately three years. While our stock-based compensation expense for stock options has not been significant to date, we expect the effect to grow in future periods due to the potential increases in the value of our common stock and increased number of stock options granted due to anticipated increases in our overall headcount.

The following table presents the grant dates of stock options that we granted from April 15, 2016 through March 31, 2017 along with the corresponding exercise price for each option grant and our current estimate of the fair value per option on each grant date, which we utilize to calculate stock-based compensation expense:

Period Granted	Number of Shares Underlying Options Granted	Exercise Price per Share	Estimated Fair Value per Share of Common Stock	Estimated Fair Value per Share of Options
November 2016	31,579	$11.07	$11.07	$7.77

At March 31, 2017, options to purchase 25,263 shares of our common stock were outstanding. The aggregate intrinsic value of these options was $, assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus. The intrinsic value of all outstanding vested and unvested options as of March 31, 2017, assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, are as follows:

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding at January 1, 2016				
Granted	31,579	$ 11.07		$ —
Exercised	—			
Cancelled or forfeited	—			
Outstanding at December 31, 2016	31,579	$ 11.07	9.7	$ 338
Granted (unaudited)	—	—		
Exercised (unaudited)	—	—		
Cancelled or forfeited (unaudited)	(6,316)	$ 11.07		
Outstanding at March 31, 2017 (unaudited)	25,263	$ 11.07	9.5	$ 540
Exercisable at December 31, 2016	—	$ —	—	$ —
Vested at December 31, 2016	—	$ —	—	$ —
Exercisable at March 31, 2017 (unaudited)	2,105	$ 11.07	9.6	$ 45
Vested at March 31, 2017 (unaudited)	2,105	$ 11.07	9.6	$ 45

Determination of the Fair Value of Common Stock on Grant Dates

As there has been no public market for our equity instruments to date, the estimated fair value of our common shares has been determined by our board of directors as of the grant date, with input from management, considering our most recently available third-party valuations of common shares and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. Following the consummation of this offering, the fair value of our common stock will be determined based on the quoted market price of our common stock. We engaged an independent third-party valuation specialist to perform contemporaneous valuations as of September 30, 2016 and May 31, 2017. The third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, or AICPA's Practice Aid. In conducting the valuations, the independent third-party valuation specialist considered all objective and subjective factors that it believed to be relevant for each valuation conducted in accordance with AICPA's Practice Aid, including our best estimate of our business condition, prospects and operating performance at each valuation date. Other significant factors included:

- the rights, preferences and privileges of our preferred stock as compared to those of our common stock, including the liquidation preferences of our preferred stock;
- our results of operations, financial position and the status of research and development efforts;
- the composition of, and changes to, our management team and board of directors;
- the lack of liquidity of our common stock;
- our stage of development and business strategy and the material risks related to our business and industry;

- the valuation of publicly traded companies in the life sciences and biotechnology sectors, as well as recently completed mergers and acquisitions of guideline companies;
- any external market conditions affecting the life sciences and biotechnology industry sectors;
- the likelihood of achieving a liquidity event for the holders of our common stock and stock options, such as an initial public offering, or IPO, or a sale of our company, given prevailing market conditions; and
- the state of the IPO market for similarly situated privately held biotechnology companies.

The dates of our contemporaneous valuations have not always coincided with the dates of our stock option grants. In determining the exercise prices of the stock options set forth in the table above, our board of directors considered, among other things, the most recent contemporaneous valuation of our common stock and their assessment of additional objective and subjective factors that were relevant as of the grant dates. The additional factors considered when determining whether any changes in the fair value of our common stock had occurred between the most recent contemporaneous valuation and the grant dates included our stage of research and preclinical development, our operating and financial performance and current business conditions.

There are significant judgments and estimates inherent in the determination of the fair value of our common stock. These judgments and estimates include assumptions regarding our future operating performance, the time to completing an IPO or other liquidity event, the related valuations associated with such events, and the determinations of the appropriate valuation methods at each valuation date. If we had made different assumptions, our stock-based compensation expense, net loss and net loss per share applicable to common stockholders could have been materially different.

Common Stock Valuation Methodologies

The valuations we obtained were prepared in accordance with the guidelines in AICPA's Practice Aid, which prescribes several valuation approaches for setting the value of an enterprise, such as the cost, market and income approaches, and various methodologies for allocating the value of an enterprise to its common stock. We generally used the market approach, in particular the guideline company and precedent transaction methodologies, based on inputs from comparable public companies' equity valuations and comparable acquisition transactions, to estimate the enterprise value of our company.

Methods Used to Allocate Our Enterprise Value to Classes of Securities

In accordance with AICPA's Practice Aid, we considered the various methods for allocating the enterprise value across our classes and series of capital stock to determine the fair value of our common stock at each valuation date. The methods considered consisted of the following:

- *Probability-Weighted Expected Return Method, or PWERM.* The PWERM is a scenario-based analysis that estimates the value per share based on the probability-weighted present value of expected future investment returns, considering each of the possible outcomes available to us, as well as the economic and control rights of each share class.
- *Option Pricing Method, or OPM.* Under the option pricing method, shares are valued by creating a series of call options with exercise prices based on the liquidation preferences and conversion terms of each equity class. The values of the preferred and common stock are inferred by analyzing these options.
- *Current Value Method.* The current value method of allocating value between security holders analyzes the current capital structure of a business as of the time the valuation. It assumes that preferred shares can be valued based upon the amount of their liquidation preferences, unless their conversion rights are "in the money." Preferred shares are "in the money" if the estimated fair value of the business is high enough that the preferred shareholder would choose to exercise their right to

convert their preferred shares into of common stock, taking into account senior liquidation preferences, multiple liquidation preferences, and participation feature.

The foregoing valuation methodologies are not the only methodologies available and they will not be used to value our common stock once this offering is complete. We cannot make assurances as to any particular valuation for our common stock. Accordingly, investors are cautioned not to place undue reliance on the foregoing valuation methodologies as an indicator of future stock prices.

Valuation of Common Stock at September 30, 2016

We engaged a third-party valuation specialist to conduct a contemporaneous valuation of our common stock at September 30, 2016. We chose the OPM to estimate our enterprise value and to allocate this value to the various outstanding equity instruments. The option pricing method of allocating value between security holders analyzes the value of each class of security by treating it as a call option on a portion of the future of a business. It assumes that a formula, such as the Black-Scholes model, can calculate the fair value, if provided with estimate of certain values. The values to be estimated are:

- Share price;
- Expiration date;
- Volatility; and
- Risk-free rate of return.

Under this method, the common share has value only if the funds available for distribution to shareholders exceed the value of the liquidation preference at the time of a liquidity event, assuming the enterprise has funds available to make a liquidation preference meaningful and collectible by the shareholders. The common share is modeled as a call option that gives the owner the right but not the obligation to buy the underlying enterprise value at a predetermined or exercise price. In the model, the exercise price is based on a comparison with the equity value rather than, as in the case of a “regular” call option, a comparison with a per-share price. Thus, common shares are considered to be call options with a claim on the enterprise at an exercise price equal to the remaining value immediately after the preferred shares are liquidated.

The option-pricing method considers the various terms of the unit holder agreements, including the level of seniority among the securities, dividend policy, conversion ratios, and cash allocations, upon liquidation of the enterprise. In addition, the method implicitly considers the effects of the liquidation preference as of the future liquidation date, not as of the valuation date. However, the method may be complex to implement and is sensitive to certain key assumptions, such as the volatility assumption.

The OPM, as applied under the Black-Scholes model, is appropriate to use when the range of possible further outcomes is so difficult to predict that forecasts would be highly speculative. That is, use of the method under Black-Scholes is generally appropriate in situations in which the enterprise has many choices and options available, and the enterprise’s value depends on how well it follows an uncharted path through the various possible opportunities.

The inputs were applied in the Black-Scholes calculations of the OPM are as follows:

- Time to liquidity: 5 years
- Expected volatility of underlying securities: 80.0%
- Risk free rate of return: 1.14%
- Dividend yield: 0.0%

Based on these input variables, the fair value of our common stock on September 30, 2016 was $11.07 per share.

Stock Options Granted in November 2016

Our board of directors granted options to purchase 31,579 common shares in November 2016 with an exercise price of $11.07 per share, which the board determined to be fair value per share of the common stock.

At the time of the grant of the options, we considered the third-party valuation as of September 30, 2016 as well as other factors in estimating the fair value of our common stock on their respective grant dates. From September 30, 2016 through November 30, 2016, we did not receive any significant scientific data or results from the development of our lead product candidate KB103, or experience any other material events that would affect the fair value of our common stock. In addition, there were no significant changes in the overall capital markets that affected the assumptions used to estimate the fair value of our common stock. Given the uncertainty around a future liquidity event our board of directors considered that no significant event or other circumstances had occurred between September 30, 2016 and November 30, 2016, and determined that there was no change in the fair value of our common stock during that period.

Results of Operations

Year Ended December 31, 2016

(in thousands)	Year Ended December 31, 2016
Revenues	
Revenues	$ —
Total revenues	—
Expenses	
Research and development	741
General and administrative	402
Total operating expenses	1,143
Loss from operations	(1,143)
Other Expense	
Interest expense, net	(7)
Total other expense	(7)
Net loss	$ (1,150)

Research and Development Expenses

Research and development expenses at December 31, 2016 were $741 thousand. The expenses incurred were primarily related to lab supplies of $173 thousand, external development of $380 thousand, consulting costs of $131 thousand and facilities related costs of $57 thousand.

General and Administrative Expenses

General and administrative expenses at December 31, 2016 were $402 thousand. The expenses incurred were primarily related to travel and related costs of $142 thousand, legal costs of $141 thousand, accounting services of $61 thousand, facilities related costs of $18 thousand, office supplies of $18 thousand, other professional and consulting of $7 thousand, other administrative of $7 thousand, and stock based compensation expense of $8 thousand.

Interest Expense, Net

Interest expense was approximately $7 thousand for the year ended December 31, 2016.

Three Months Ended March 31, 2016 and March 31, 2017

(in thousands)	Three Months Ended March 31, 2016	2017	Change
	(unaudited)		
Revenues			
Revenues	$ —	$ —	$ —
Total revenues	—	—	—
Expenses			
Research and development	—	319	319
General and administrative	—	146	146
Total operating expenses	—	465	465
Income (loss) from operations	—	(465)	(465)
Other Expense			
Interest expense, net	—	(29)	(29)
Total other expense	—	(29)	(29)
Net loss	$ —	$ (494)	$ (494)

There is no financial activity presented in the first quarter of 2016 because we did not commence operations until April 15, 2016, and as a result, there is no activity in the statement of operations for the three months ended March 31, 2016.

Research and Development Expenses

Research and development expenses for the three months ended March 31, 2017 were $319 thousand. The expenses incurred were primarily related to external development of $171 thousand, consulting costs of $57 thousand, lab supplies of $66 thousand and facilities related costs of $25 thousand.

General and Administrative Expenses

General and administrative expenses for the three months ended March 31, 2017 were $146 thousand. The expenses incurred were primarily related to travel and related costs of $39 thousand, accounting services of $17 thousand, legal costs of $17 thousand, stock based compensation costs of $59 thousand and other administrative costs of $14 thousand.

Interest Expense, Net

Interest expense was $29 thousand for the three months ended March 31, 2017.

Liquidity and Capital Resources

Overview

Since our inception and through March 31, 2017, we have received an aggregate of $1.4 million in gross proceeds from the issuance of equity securities and an aggregate of $2.1 million from debt financings. At March 31, 2017 our cash was approximately $2.0 million.

Debt Financings

On November 16, 2016, we executed a note purchase agreement under which convertible promissory notes were issued. Each note bears interest at a rate of 6% per annum, which is accrued based on a 365 day year and mature on May 14, 2018, unless sooner paid or converted. The notes become immediately due and payable in the event of an occurrence of default by us. As of December 31, 2016 and March 31, 2017,

the outstanding balance of the notes was $1.8 million and $2.1 million, respectively. In the event the Company sells, merges, consolidates or reorganizes, sells stock through a public offering, then all of the outstanding notes, at the option of the holder, become immediately due and payable or convert into a number of shares of common stock or common units.

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.

Based on our planned use of the net proceeds of this offering and our existing cash resources, we believe that our available funds following this offering 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.

Cash Flows

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

	Year Ended December 31,	Three Months Ended March 31,	
	2016	2016	2017
Net cash used in operating activities	$ (1,311)	$ —	$ (257)
Net cash used in investing activities	(15)	—	(7)
Net cash provided by financing activities	3,249	100	300
Net increase in cash	$ 1,923	$ 100	$ 36

Operating Activities

Net cash used in operating activities was $1.3 million for the year ended December 31, 2016 and consisted primarily of a net loss of $1.2 million adjusted for non-cash items including nominal depreciation expenses, stock-based compensation expense of $33 thousand, and a net increase in operating assets and liabilities of approximately $194 thousand.

Net cash used in operating activities was $257 thousand for the three month ended March 31, 2017 consisted primarily of a net loss of $494 thousand adjusted for non-cash items including nominal depreciation expenses, stock-based compensation expense of $91 thousand, and a net decrease in operating assets and liabilities of approximately $146 thousand.

Investing Activities

During the year ended December 31, 2016, our investing activities used net cash of $15 thousand. The use of net cash resulted from purchases of laboratory property and equipment.

During the three months ended March 31, 2017, our investing activities used net cash of approximately $7 thousand. The use of net cash resulted primarily from purchases of laboratory property and equipment.

Financing Activities

Net cash provided by financing activities was $3.2 million for the year ended December 31, 2016, which consisted of $1.4 million from the issuance of preferred stock and $1.8 million from the issuance of convertible promissory notes.

Net cash provided by financing activities was $300 thousand for the three months ended March 31, 2017, which was from the issuance of convertible promissory notes.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements.

Net Operating Loss Carryforwards

From our inception through December 31, 2016, we were organized as a California limited liability company, or LLC, for federal and state income tax purposes, and therefore, all items of income or loss

through December 31, 2016 flowed through to the members of the LLC. Effective January 1, 2017, we converted from an LLC to a Delaware C-corporation for federal and state income tax purposes. Prior to the conversion to a C-corporation, we did not record deferred tax assets or liabilities or have any net operating loss, or NOL, carryforwards for federal income tax purposes. Effective upon our conversion to a C-corporation, we became subject to income tax at the federal and state levels. Accordingly, as of March 31, 2017 (unaudited), we recorded a deferred tax asset for federal and state income taxes, which consists primarily of NOL carryforwards and research & development credit, as defined by the Internal Revenue Service.

We did not record a current or deferred income tax expense or benefit for the year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited). Because we were an LLC for the year ended December 31, 2016, we were not subject to federal income tax. A reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate of 40 percent for the year to income tax expense (benefit) as reflected in our financial statements for the three months ended March 31, 2017 (unaudited) is as follows:

	March 31, 2017 (unaudited)
Federal income tax expense (benefit) at statutory rate	$ (197)
Change in valuation allowance	201
Other non-deductible expenses	2
Research & development credit	(6)
Others	—
Total tax expense (benefit)	$ —

The significant components of our deferred tax assets as of March 31, 2017 (unaudited) are as follows:

	March 31, 2017 (unaudited)
Deferred tax assets:	
Net operating loss carryforwards	$ 163
Non-qualified option	36
Research & development credit	6
Depreciation	(4)
Total deferred tax assets	201
Valuation allowance	(201)
Net deferred tax assets	$ —

We have evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on our history of operating losses, we have concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, we have provided a full valuation allowance for deferred tax assets as of March 31, 2017. The valuation allowance increased approximately $201 thousand during the three months ended March 31, 2017, due primarily to the federal and state net operating losses generated during the period.

As of December 31, 2016, we had no U.S. federal NOL carryforwards because the Company was organized as a flow-through entity. As of March 31, 2017, we had U.S. federal NOL carryforwards of approximately $163 thousand which may be available to offset future income tax liabilities and expire at various dates through 2037.

Under the provisions of the Internal Revenue Code, the NOL carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest

of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of our company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. We have completed financings since inception which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

We file income tax returns in the United States at the federal level and in states in which we conduct business activities. The federal and state income tax returns are generally subject to tax examinations for the tax year ended December 31, 2016. To the extent we have tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state tax authorities to the extent utilized in a future period.

Contractual Obligations

The following table summarizes our outstanding contractual obligations as of payment due date by period at December 31, 2016 (in thousands):

	Total	Less Than 1 Year	Years 1-3	Years 4-5	More Than 5 Years
Future minimum operating lease payments(1)	$ 191	$ 100	$ 91	$ —	$ —
Less: minimum payments to be received from non-cancelable subleases	(39)	(22)	(17)	—	—
Total minimum lease payment, net	$ 152	$ 78	$ 74	—	—
Obligation to contract manufacturing organization	$2,011	$ 1,511	$ 500	$ —	$ —

(1) In May 2016, we leased office and laboratory space at 2100 Wharton St., Pittsburgh, PA that expires in October 2018.

Qualitative and Quantitative Disclosures About Market Risk

Our cash is held in an operating account as of March 31, 2017. 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.

JOBS Act

In April 2012, the JOBS Act was enacted in the United States. Section 107 of the JOBS Act provides that an “emerging growth company” can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. Thus, an emerging growth company can 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 extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth public companies.

BUSINESS

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 a patent pending engineered 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 the 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 to expand our pipeline to include other dermatological indications in the future.

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 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. We estimate that there are presently 3,200-3,500 diagnosed DEB patients in the world, with the majority of such patients in the European Union and United States, and lesser populations in Japan and Canada.

We believe our approach to treating DEB with KB103 is novel. The current standard of care for DEB patients is limited to palliative measures which seek to provide relief from some of the symptoms of DEB but do not meaningfully impact disease outcomes. Other known efforts to develop DEB treatments are employing autologous approaches to creating therapeutic products. Autologous treatments use a patient’s own tissues and cells to manufacture an individualized therapy. Such therapies are expensive, invasive and time consuming to use, and require highly sophisticated medical teams and procedures. In contrast, KB103 is designed to be an off-the-shelf treatment for DEB that can be applied either intra-dermally or topically to a patient’s skin, every three to four months. Unlike the current standard of care, KB103 seeks to treat DEB at the molecular level through gene therapy, and is intended to be a non-invasive treatment that can be used without requiring hospitalization or individually customized treatment.

KB103 is a replication-defective, non-integrating viral vector that has been engineered employing our STAR-D platform to deliver functional human COL7A1 genes directly to the patients’ dividing and non-dividing skin cells. We believe our STAR-D platform is an optimal approach to treating dermatological conditions due to the nature of the HSV-1 viral vector we have created. Our viral vector has a natural affinity for skin epithelium and we believe it can penetrate skin cells more efficiently than other viral vectors used in gene therapy. In addition, unlike many other gene therapy treatments, our viral vector is non-integrating, meaning it does not carry the potential risk of disrupting essential host genes and potentially triggering oncogenesis, because the vector and its genes remain physically separate from the host cell chromosome. Our viral vector also has a high payload capacity relative to most vectors, so it can accommodate large genes like COL7A1 and may allow us to insert multiple genes and other effectors in the future. This ability of our STAR-D platform could allow for the potential treatment of multi-factoral dermatological conditions like psoriasis and chronic wounds.

Preclinical studies evaluating intradermal and topical delivery of KB103 have been completed, and have demonstrated successful introduction of a functional COL7A1 gene to the host cells and subsequent

expression of COL7. Additional studies to determine the durability, efficacy and dosing frequency using surrogate models of DEB are currently ongoing. We are presently completing a Good Laboratory Practice, or GLP, toxicity study and intend to file an Investigational New Drug, or IND, application for KB103 with the FDA in January 2018.

Our management team includes individuals with expertise in gene therapy, product development, and manufacturing and commercialization in the biotechnology space. In addition, our management team members have successful track records in shepherding drugs from research to product approval. Suma M. Krishnan, our founder and Chief Operating Officer, has 25 years of drug development experience and led the discovery, development and approval of Vyvanse, a blockbuster drug to treat Attention Deficit Hyperactivity Disorder, or ADHD. She was also a member of the regulatory teams for both Adderall XR and Fosrenol, providing key support in moving both products toward approval, and more recently advanced several gene therapy programs from discovery to clinical development. Our Chairman and Chief Executive Officer, Krish S. Krishnan, brings 20 years of broad leadership and management experience in the biopharmaceutical industry. Our scientific team collectively has over 20 years of experience in HSV engineering and purification, providing the expertise needed to successfully optimize our HSV-1 vector production process. In addition, we are guided by key opinion leaders, or KOLs, who are generally accepted in the medical and scientific communities to be leading experts in the DEB and orphan dermatological disease space. Our KOLs include Dr. Peter Marinkovich of the Department of Dermatology of Stanford University and Dr. Andrew South of the Department of Dermatology of Thomas Jefferson University.

Our success also depends in part on our ability to protect our intellectual property, including the STAR-D platform. We have adopted a strategy of seeking patent protection in the United States and abroad where appropriate with respect to certain of our technologies relating to our products and process. As of June 9, 2017, we are actively prosecuting a patent application in front of the USPTO directed to our products and processes related to the treatment of DEB, and a corresponding international patent application has been filed in accordance with the Paris Cooperation Treaty. Additionally, a patent application has been filed with the USPTO seeking protection for our core STAR-D viral platform technologies. We continue to actively develop our portfolio through the filing of new patent applications, divisionals and continuations relating to our technologies as we deem appropriate.

Our Strengths

We believe we are the first biotechnology company to seek to use gene therapy to develop products for dermatological indications that can be used without requiring individually customized treatment, which we refer to as an “off-the-shelf” treatment. We believe our organization and technology benefit from a singular set of strengths that will allow us to create and establish a leadership position in developing gene therapy treatments for dermatological indications. These strengths include:

- A first mover advantage in dermatological gene therapy with regards to:
  - An off-the-shelf gene therapy product candidate, and
  - Topical gene therapy application;
- Our STAR-D platform, a proprietary, integrated gene therapy platform comprised of a library of optimized HSV-1 vectors and their complimenting cell lines with higher and more durable expression, lower immune response and improved manufacturability when compared with other viral vectors currently being used in dermatological gene therapy;
- The significant affinity to the skin and high payload capacity of our HSV-1 viral vector, which will allow us to deliver single and multiple genes to treat orphan and other dermatological indications;
- A proprietary process for both upstream (vector production) and downstream (purification) portions of the manufacturing process, which positions us to maximize scalability, quality and reliability;

- A scientific team with expertise in the HSV-1 viral vector; and
- A management team with a track record in developing drugs from research to approval.

Our Strategy

Our objective is to become a leader in developing and commercializing gene therapy treatments for dermatological indications. The strategy we intend to employ to accomplish this objective is to initially focus on dermatological indications in the rare or orphan disease space, build out and leverage our in-house manufacturing capabilities, and then leverage our STAR-D platform to develop, manufacture and commercialize treatments for non-orphan indications within dermatology.

- **Why dermatology**—We believe the characteristics of our STAR-D platform are ideal for application in dermatology because of HSV-1's high tropism, or natural affinity, for skin cells. This allows the viral vector to penetrate skin cells more efficiently than other gene therapy vectors, and makes topical delivery of gene therapy possible. Dermatology is also attractive because treatments for diseases affecting the skin have clearly defined, objective clinical endpoints with validated measurement tools that are accepted by the FDA. We believe these clearly defined endpoints will help accelerate the process of clinical development and regulatory approval for our dermatological products.
- **Why rare or orphan diseases**—We believe there is significant unmet medical need for rare or orphan diseases, because the patient populations are not sizeable enough to attract the attention of large commercial entities. In contrast, we believe there are advantages to developing in this space, including that these diseases are genetic and frequently affect children, and so have been studied extensively and have concentrated, supportive networks of KOLs and patient advocacy groups. We have established strong relationships with such KOLs which we believe will aid us in obtaining data more rapidly and assist with the development and regulatory approval process. In addition, rare and orphan diseases, particularly monogenic ones like DEB, have defined clinical endpoints that have been validated by the FDA. There are also regulatory designations such as the FDA's orphan drug designation, breakthrough drug designation, fast track drug designation and rare pediatric disease designation, one or more of which we believe, if successfully obtained, can provide certain regulatory and commercial advantages and incentives for developing treatments in this space. If we are able to successfully achieve one or more of these designations, we believe this will aid in the commercialization of our product candidates.
- **How we will manufacture**—While we currently outsource our manufacturing, we believe there is value in maintaining control of our entire manufacturing process. We intend to continue to devote substantial resources to developing the STAR-D platform and bringing our scalable manufacturing process in-house. Because of the high demand for contract manufacturing in gene therapy, we believe the focus on expanding the STAR-D platform and establishing current good manufacturing practices, or cGMP, manufacturing in-house will ultimately accelerate the process of regulatory approval for our products.
- **How we can expand**—We believe we can eventually expand beyond rare and orphan diseases by leveraging our STAR-D platform and expertise in viral vector selection and design, physical vector delivery and vector manufacturing to pivot into other gene therapy treatments for dermatological applications. For example, we believe that the large payload capacity of the viral "backbone" in the STAR-D platform will allow us to deliver multiple genes and other effectors using the platform and afford us an opportunity to treat non-monogenic diseases like psoriasis, as well as conditions which are not necessarily the result of an inherited genetic defect, such as chronic wounds. If we are able to successfully develop and commercialize products to treat non-orphan dermatological diseases, we intend to seek collaborative alliances towards commercializing these therapies among the broader population of patients in these indications.

Background on Gene Therapy

Many diseases have a genetic aspect whereby a mutated gene linked to a disease is passed down from generation to generation. Genes produce proteins that perform a vast array of functions within all living organisms, through a process called gene expression. A mutation, or alteration, in the gene or in sequences that control the expression of that gene can cause aberrations in intracellular protein production, which can cause disease. Gene therapy seeks to introduce a functional copy of the defective gene into a patient's own cells, a process called gene transfer. Gene therapy thereby has the potential to change the way patients are treated by correcting the underlying genetic defect that is the cause of their disease, rather than offering solutions that only address their symptoms.

In the gene transfer process, a functional gene is delivered and incorporated into a patient's cells through a delivery system called a vector, which are most commonly based on naturally occurring viruses that have been modified to take advantage of the virus' natural ability to introduce genes into cells. However, unlike naturally occurring viruses, which replicate following infection of a target cell and have the capacity to infect new cells, our viral vectors are modified to be non-replicating by deleting that portion of the viral genome responsible for replication. Gene transfer using a viral vector is called transduction, and the resulting gene-modified cells are described as transduced cells.

A growing body of gene therapy-based clinical data, the establishment of regulatory guidelines to govern the development and approval of gene therapy products and increased investment from the biopharmaceutical industry suggest that gene therapy is being increasingly accepted as an important new therapeutic modality for patients with unmet medical need. We believe that if successful, our lead product candidate, KB103, together with the STAR-D platform, will help bring gene therapy to the treatment of dermatological indications.

Our STAR-D Gene Therapy Platform

We believe that certain inherent features of the HSV-1 virus, combined with our ability to strategically modify the virus in the form we employ as our gene delivery backbone, provides our proprietary gene therapy platform, which we refer to as the Skin TARgeted Delivery platform, or STAR-D platform, with several advantages over other viral vector platforms for use in dermatological applications, including the following:

- **Non-Integrating Nature**: Conceptually our STAR-D platform is similar in its simplicity, safety, and ease of use to other non-integrating gene therapy platforms like Adeno-Associated Virus, or AAV. Upon entry into cells, the HSV-1 vector persists as an episomal unit in the nucleus, remaining physically separate from the host cell chromosome. Other vectors we are aware of currently being used in the development of gene therapy treatments for dermatological conditions, such as lentiviral and retroviral vectors, integrate into the host cell DNA in order to achieve gene expression. Integration into the host cell DNA carries the risk of disrupting essential host genes, and consequently leads to a risk of oncogenesis. In contrast, a non-integrating vector such as our HSV-1 vector does not carry the potential risk of oncogenicity due to random integration into the DNA.
- **Payload Capacity**: HSV-1 is a large virus, approximately 150 kilobase, or Kb, pairs of DNA in size. We have made strategic deletions within this genome to remove critical "immediate early", or IE, genes. These IE genes are required for expression of most of the downstream genes that allow the HSV-1 virus to replicate and destroy host cells. Deletion of these IE genes inhibits expression of most of the viral proteins, making the resulting viral vector replication-deficient and non-toxic. These deletions also enable the vector to easily accommodate a payload of 30Kb or greater without any significant impact on yield or titer. In KB103, we have successfully inserted two functional copies of the complete ~9Kb human COL7A1 gene, thereby providing an off-the-shelf treatment for this debilitating disease. In contrast, packaging capacity for most other viral vectors being used in dermatological indications is under 8Kb which limits their ability to package large genetic materials. In addition, we believe the high payload capacity of our viral vector will allow us to insert

multiple genes and other effectors, allowing for the potential treatment of non-monogenic dermatological conditions such as psoriasis and chronic wounds.

- **Skin Tropism**: Poor infection of skin epithelia has remained a major hurdle for direct delivery of most viral vectors. HSV-1 has a natural affinity, or tropism, for the skin epithelium; therefore our viral vector penetrates skin cells much more efficiently than other viral vectors, resulting in transduction efficiencies as high as 95% in cell-based studies.

  This transduction efficiency, along with the high payload capacity of our vector discussed above, are responsible for the high levels of transgene expression in animal models. In addition, these factors are critical contributors to our ability to create an off-the-shelf gene therapy treatment where others are taking autologous approaches. Because the genes that cause many dermatological diseases are quite large, many of our competitors can only fit a single gene, or in some cases may need to manipulate the genetic material in order to fit the limited payload capacity of their vectors. From our review of published research, we estimate that some of these autologous gene therapy approaches may have transduction efficiencies as low as 10% in skin. In order to develop an effective treatment in the face of payload capacity and transduction limitations, they may need to introduce the therapeutic gene into a patient's tissues or cells ex vivo to create an individual treatment, which is re-administered back to the patient once the gene-modified tissues have achieved a sufficient level of gene expression. The greater payload capacity of our vector and the high transduction efficiencies achieved allow us to deliver a full gene directly to any patient's tissues for in vivo gene expression without additional manipulation.

- **Low Immunogenicity**: One of the major challenges with other viral vector platforms is that the host immune system may recognize them as foreign bodies and launch a robust immune response, resulting in toxicity and rapid removal of the virus. Wild type HSV-1 is known to persist in the body by becoming latent and hiding from the immune system in the cell bodies of neurons. We have harnessed the natural ability of HSV-1 to evade host-mediated immunogenicity, while removing specific viral elements that exacerbate the host immunity, thus making the viral vector safer and allowing for repeat administration as needed to achieve durability of effect.

- **Stability**: HSV-1 is extremely stable and resistant to degradation by shear, solvents and enzymes, facilitating purification and final formulation of our product candidates. These characteristics of HSV-1 provide a stability advantage to our KB103 product candidate. Although frozen for long-term storage, it is also stable under refrigerated conditions for short-term storage and shipment, and stable over several freeze-thaw cycles.

- **Reproducible Manufacturing and Scalability**: Successful production of viral vectors involves two steps: (i) the 'upstream' process, which yields a bulk virus harvest; and (ii) the 'downstream' process, which involves purification and concentration of the clinical product. Successful and reproducible execution of both processes is critical for clinical manufacturing and scale-up. Our scientific team collectively has over 20 years of experience and expertise in HSV engineering and purification that has allowed us to successfully optimize our HSV-1 vector production process.

- **Existing Regulatory Precedent**: The first FDA-approved oncolytic virus product, Imlygic® by Amgen, is based on a genetically engineered HSV-1 virus. To our knowledge, this is the only FDA-approved viral vector-based drug to date. Because this product also employs an HSV-1 backbone it has created a regulatory precedent for approval of an HSV-1-based therapy. In addition, Imlygic® is a chronic therapy, given bi-weekly, which provides support for the use of an HSV-1 backbone in chronic gene therapy of the type we are developing.

Background on Dystrophic Epidermolysis Bullosa

Dystrophic epidermolysis bullosa, or DEB, belongs to a group of genetic skin diseases known more broadly as epidermolysis bullosa, or EB, characterized by genetic defects of structural proteins of the skin,

resulting in skin fragility and the formation of blisters. Blisters form as a result of rubbing, trauma or even in some cases from slight contact such as a simple hug. The subtypes of EB are distinguished by the location of the blister in the skin. DEB is associated with mutations in the gene coding for type VII collagen, or COL7, a major component of the anchoring fibrils which anchor the top layer of skin, called the epidermis, to an underlying layer, called the dermis, and provide structural adhesion in a normal individual. The lack of COL7 in DEB patients causes blisters to occur in the dermis as a result of separation from the epidermis. Genetic linkage studies have identified COL7A1 as the gene responsible for DEB. Over 200 distinct mutations in COL7A1 have been identified in DEB patients.

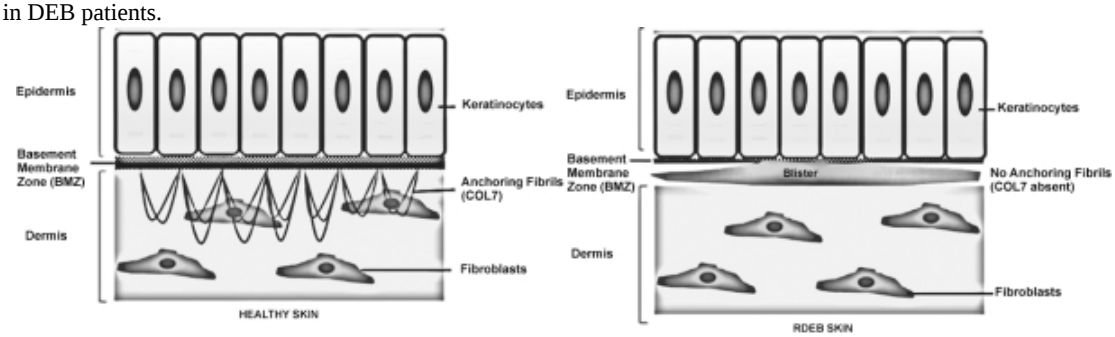

Figure 1. Healthy skin vs. DEB patient skin

The most severe form of DEB is recessive DEB, or RDEB, in which both COL7 and anchoring fibrils are severely diminished in the patient's skin due to null mutations in the COL7A1 gene. As a result, RDEB is characterized by severe skin blistering, extremely fragile skin, mutilating scarring of the hands and feet, joint contractures, strictures of the esophagus, and often, eventually the development of aggressive squamous cell carcinomas which may shorten the patient's life. DEB also occurs in the form of dominant DEB, or DDEB, which is considered to be a more mild form of DEB. In DDEB blistering is often limited to the hands, feet, knees, and elbows, and often improves somewhat with age.

Currently, there is no effective therapy for any form of DEB, and RDEB patients have a low life expectancy. Nearly 10% of RDEB patients die before the age of 10, almost 40% die by the age of 20, and over 70% die before the age of 30. Persistent blistering begins at birth and contributes to the high mortality risk due to bacterial infection. In a study of 41 RDEB patients, the infectious causes of pneumonia and sepsis resulted in death in close to 15% and 10% of cases, respectively. Patients who survive bacterial sepsis during early infancy are at a high risk of later developing more severe complications such as growth retardation due to gastrointestinal involvement, multifactorial anemia, esophageal strictures, corneal scarring and/or progressive blindness, renal failure, progressive hand and foot deformities, muscle contractures that restrict movement, anemia, esophageal strictures, rapid tooth decay, nail deformities, and hair loss. The onset of aggressive squamous cell carcinoma, sepsis or malnutrition due to an inability or unwillingness to eat due to mouth or esophagus involvement, may also result in death among these patients.

Existing Treatments for DEB

At present, there is no FDA- or EMA-approved treatment for DEB. The management of DEB currently consists of palliative care, which generally consists of prevention of mechanical forces that produce new blisters, wound care, nutritional support, and infection control, all of which help treat the symptoms but not the causes of DEB. Wound care usually includes treatment of new blisters by lancing and draining. Wounds are then dressed with a non-adherent material, covered with padding for stability and protection, and secured with an elastic wrap for integrity. Due to the increased risk of bacterial resistance, topical antibiotic ointments and antimicrobial dressings are typically reserved for those wounds that are colonized with bacteria and fail to heal, referred to as "critical colonization."

Individuals with DEB have increased caloric and protein needs due to the increased energy utilized in wound healing. Involvement of the digestive system in RDEB may limit nutritional intake. Infants and children with RDEB may require nutritional support, including a gastrostomy feeding tube. Anemia is typically treated with iron supplements and transfusions as needed. Other nutritional supplements may include calcium, vitamin D, selenium, carnitine and zinc.

Surveillance is important for individuals with DEB. Biopsies of abnormal-appearing wounds that do not heal may be recommended in some types of DEB due to predisposition to squamous cell carcinoma, beginning in the second decade of life. Screening for deficiencies of iron, zinc, vitamin D, selenium, and carnitine is typically recommended after the first year of life. Routine echocardiograms are recommended to identify dilated cardiomyopathy, and bone mineral density studies are recommended to identify osteoporosis. It is also typically recommended to avoid activities and bandages (including all adhesives) that may traumatize the skin.

Our Lead Product Candidate: KB-103 for the Treatment of DEB

Our gene therapy approach uses a modified HSV-1 vector designed to deliver fully functional COL7A1 gene into the patient's skin cells. Upon direct delivery to the skin, KB103 can efficiently transduce both keratinocytes and fibroblasts. Following entry of KB103 into the cell, the drug is transported down microtubules to the nucleus, and the viral genome is deposited into the nucleus. Once in the nucleus, it recruits the host cellular machinery to initiate transcription of COL7A1. The COL7A1 transcripts allow for production of a precursor protein, Procollagen 7, that is secreted by the cell and processed by enzymes to remove extra protein segments from the ends. Once these molecules are processed, they arrange themselves into long, thin bundles of mature COL7 that form anchoring fibrils. The anchoring fibrils hold the epidermis and dermis together and are essential for maintaining the integrity of the skin.

The high payload capacity of the HSV-1 vector allows us to insert two copies of the COL7A1 gene into each viral vector backbone, facilitating high expression of Procollagen 7. Current autologous therapies in development for DEB use lentivirus or retrovirus which have limited payload capacity and low transduction efficiencies. In addition, these viral vectors can target either keratinocytes or fibroblasts for gene delivery, but not both. In order to develop an effective treatment in the face of these limitations, autologous approaches treat the patient's tissues or cells ex vivo for re-administration once sufficient gene expression is achieved, which leads to an extremely expensive, invasive and time consuming treatment requiring highly sophisticated medical teams and procedures. We believe that the limited payload capacity of lentivirus and retrovirus along with low transduction efficiencies will make it very difficult, if not impossible, to develop off-the-shelf gene therapies for DEB with these viral vectors. In contrast, KB103 can transduce not only keratinocytes and fibroblasts but also all skin cells that it comes in contact with to produce optimum therapeutic levels of secreted COL7 protein. It has been shown in clinical and non-clinical studies that functional and durable replacement of COL7 protein is necessary and sufficient to correct the debilitating skin disease that inflicts these patients.

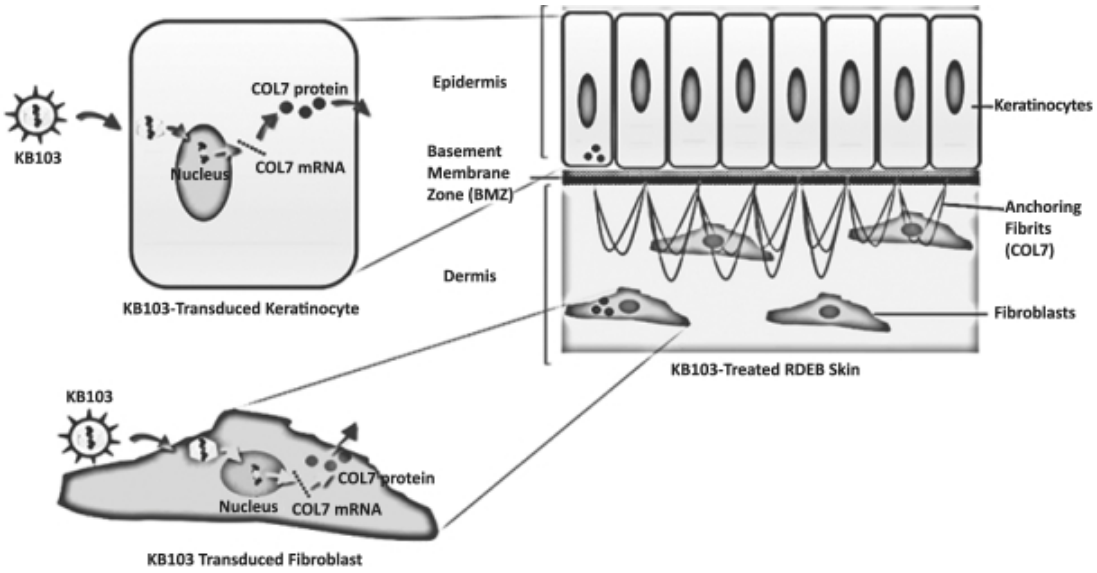

Figure 2. KB103 transduces keratinocytes and fibroblasts, supplying functional COL7

The persistence of KB103 in the skin depends on the rate of turnover of the cells where it resides. The vector has been engineered to be able to evade the host immune response and persist for the life of the cell. Additionally, we have also optimized its design to be safe and nontoxic to both dividing and non-dividing cells so that it can be reapplied as often as required.

Beyond the advantages surrounding the mechanism of action of KB103, it also has practical advantages as a therapy over autologous approaches. We believe that the major drawbacks of autologous therapies are the need for highly trained dermatologists, the high cost of treatment and the need for sophisticated equipment setup, including possible hospitalization. Additionally, because autologous treatments require time-consuming processing of a patient's own cells and tissues, there can be a significant lag of six months or more between diagnosis and commencement of treatment. As a result, we believe an off-the-shelf, non-invasive treatment such as KB103 will, if successful, be an effective alternative for treating DEB.

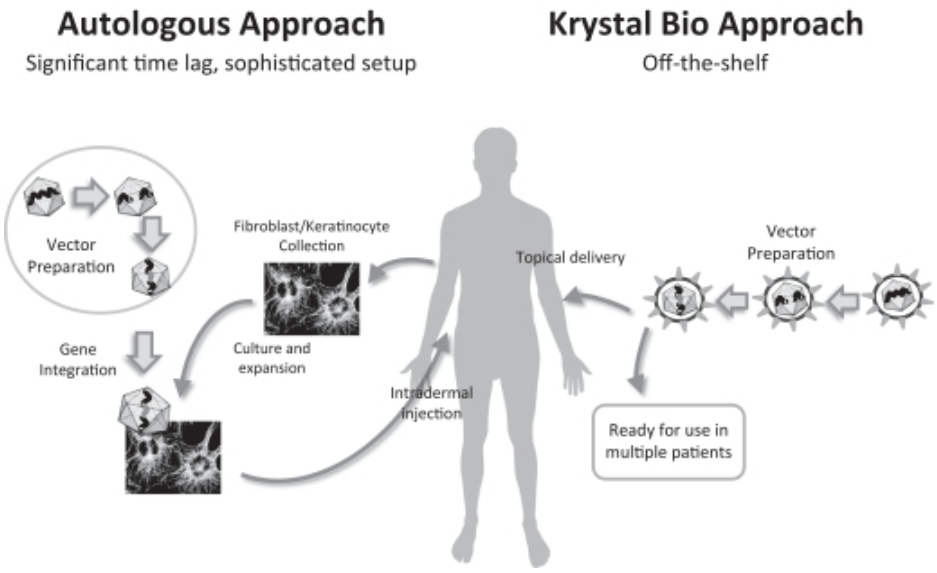

Figure 3. Krystal's approach compared to autologous therapies under development

Preclinical Proof of Concept for KB103

We have performed several preclinical tests and animal studies on KB103 to date that suggest the following:

- **Robust levels of COL7 in KB103 administered RDEB patient-derived fibroblasts**. KB103 was evaluated for transduction efficiency, expression and functionality in 2-D and 3-D cell-based assays using normal and RDEB patient-derived fibroblasts, or HDF. RDEB patient HDFs were infected with Multiplicity of Infection, or MOI, of 0.3, 1, and 3 viral particles per cell of KB103 to evaluate vector dose-dependent expression and toxicity. Transduction efficiency and COL7 expression were evaluated 48 hours post-infection using immunofluorescence, or IF, microscopy to determine COL7 localization and cellular expression. Expression was confirmed using Western Blot, or WB, analysis to evaluate total COL7 protein levels in whole cell lysates, and qRT-PCR analysis to evaluate COL7A1 transcript levels. As shown in the figure below, we detected high levels of COL7 expression at all three doses in fibroblasts administered with KB103 compared with control that did not have any KB103 administration.

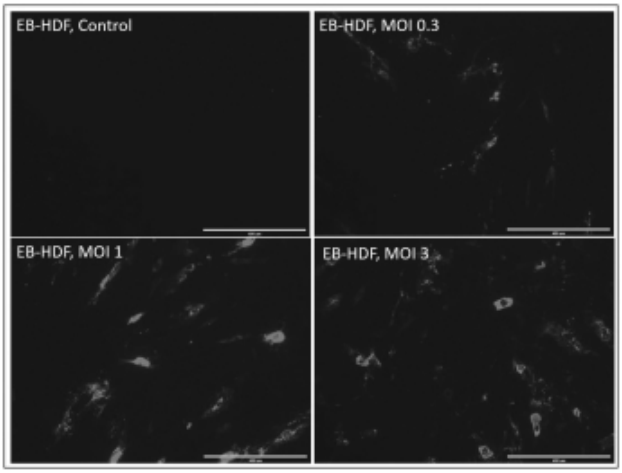

Figure 4. KB103 expresses COL7 in fibroblasts

- **Robust levels of COL7 in KB103 administered RDEB patient-derived keratinocytes**. KB103 was evaluated for transduction efficiency, expression and functionality in 2-D and 3-D cell-based assays using normal and RDEB patient-derived keratinocytes, or HDKs. RDEB patient HDKs were infected with MOI of 0.3, 1, and 3 viral particles per cell of KB103 to evaluate vector dose-dependent expression and toxicity. Transduction efficiency and COL7 expression were evaluated 48 hours post-infection using IF microscopy to determine COL7 localization and cellular expression. Expression was confirmed using WB analysis to evaluate total COL7 protein levels in whole cell lysates, and qRT-PCR analysis to evaluate COL7A1 transcript levels. As shown in the figure below, we detected high levels of COL7 expression at all three doses on keratinocytes administered with KB103 compared with control that did not have KB103 administration.

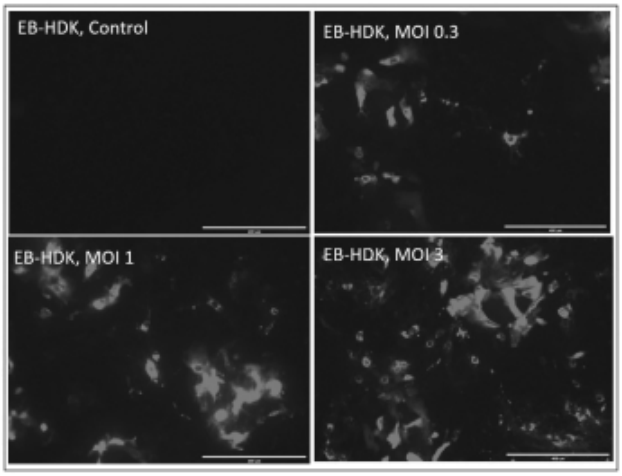

Figure 5. KB103 expresses COL7 in keratinocytes

- **Human COL7 expressed by KB103 in fibroblasts and keratinocytes is functionally active**. COL7 expressed by KB103 was confirmed to be functionally active as it increased the expression of an enzyme, Lysyl Hydroxylase 3, or LH3, in RDEB keratinocytes as shown in the figure below. LH3 aids in post-translational modification of COL7 to form anchoring fibrils. Glyceraldehyde-r Phosphate Dehydrogenase, or GAPDH, a cellular protein whose expression should not be impacted in RDEB skin due to COL7 loss, was utilized as a 'loading' control for relative quantitation of protein levels and to ensure that the same amount of cellular lysate was added to each well.

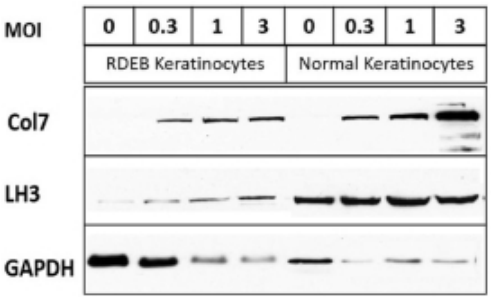

Figure 6. COL7 expressed by KB103 is functionally active

Functional COL7 is also known to inhibit production of Thromospondin-1, or TSP-1, an adhesive protein which is upregulated in the stroma of squamous cell carcinoma patients, and a marker for poor prognosis in RDEB patients who have developed squamous cell carcinoma. As seen in the figure below, COL7 expressed by KB103 inhibited TSP-1 production in a dose-dependent manner, further confirming functional activity.

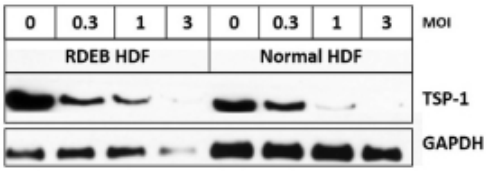

Figure 7. COL7 expressed by KB103 inhibited TSP-1 production.

Functionality was also confirmed based on preliminary plate-based assays evaluating adhesion of KB103-treated RDEB keratinocytes to type 1 collagen and fibronectin. As evidenced by the graphs below, keratinocytes expressing COL7 bind to type 1 collagen and fibronectin in a KB103 dose-dependent manner.

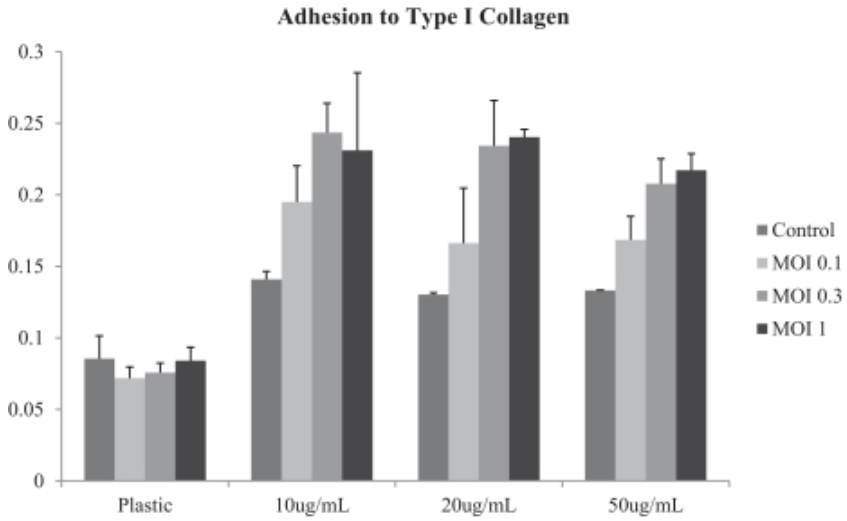

Figure 8. Adhesion to Type 1 Collagen.

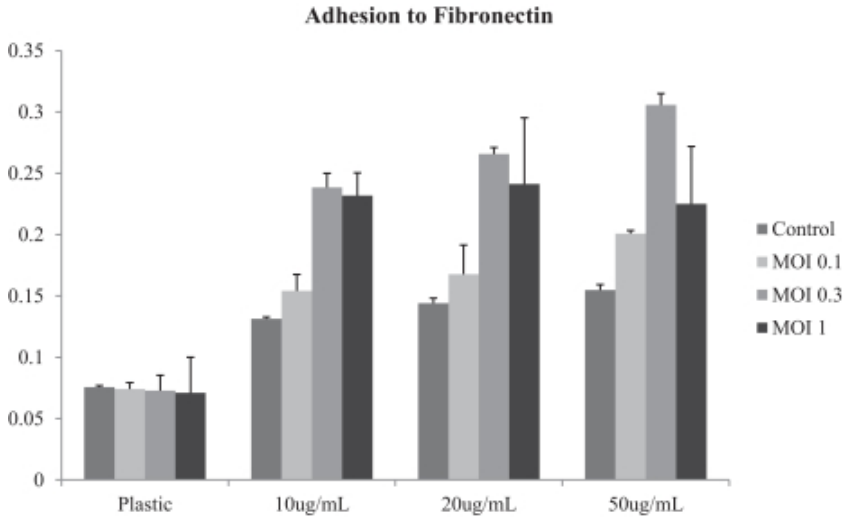

Figure 9. Adhesion to Fibronectin.

- **Confirmation of human COL7 in 3-D organotypic cultures**. Confirmation of expression and functionality in 2-D assays prompted assessment in 3-D organotypic cultures composed of RDEB fibroblasts and keratinocytes transduced with KB103. We believe that our 3-D organotypic culture study is the first study to show that KB103 infects both keratinocytes and fibroblasts and secretes COL7 from the dermis and the epidermis to optimize deposition of COL7 at the basement membrane zone, or BMZ.
- **Confirmation of COL7 expression in animal studies**. Immunocompetent mice were administered KB103 via intradermal injection to evaluate expression of COL7. Human COL7 was detected in the mouse skin and also in the BMZ.

- **Repeat administration of KB103 boosts COL7 levels**. KB103 was administered to mice on Day 1 and Day 5 and COL7 expression was assessed after one week. As seen from the graph below, levels of COL7 expression were higher after one week following repeat administration.

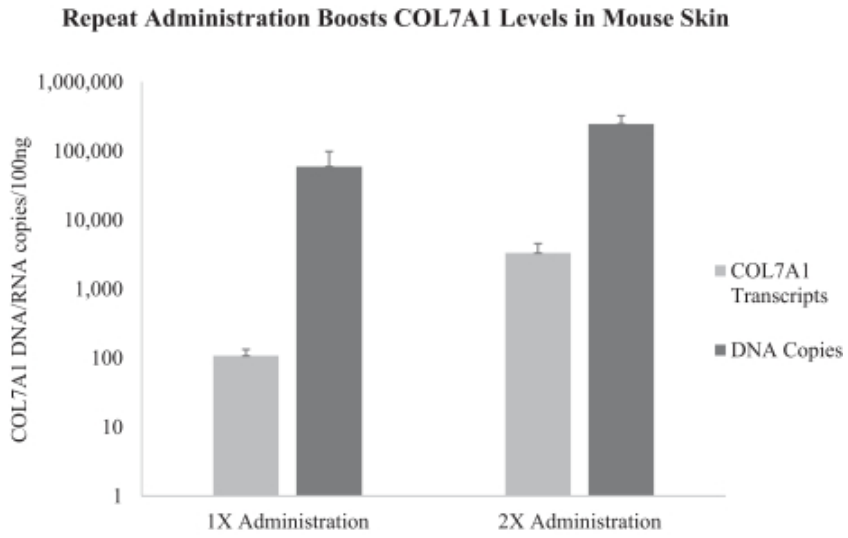

Figure 10. Repeat Administration Boosts COL7A1 Levels in Mouse Skin

- **Comparable expression levels of KB103 following intradermal and topical administration**. Efficacy of intradermal delivery vs. topical delivery to intact abraded skin was evaluated in immunocompetent mice one week after administration. As shown in the figure below, both routes of administration yielded similar COL7A1 RNA and DNA levels in the skin.

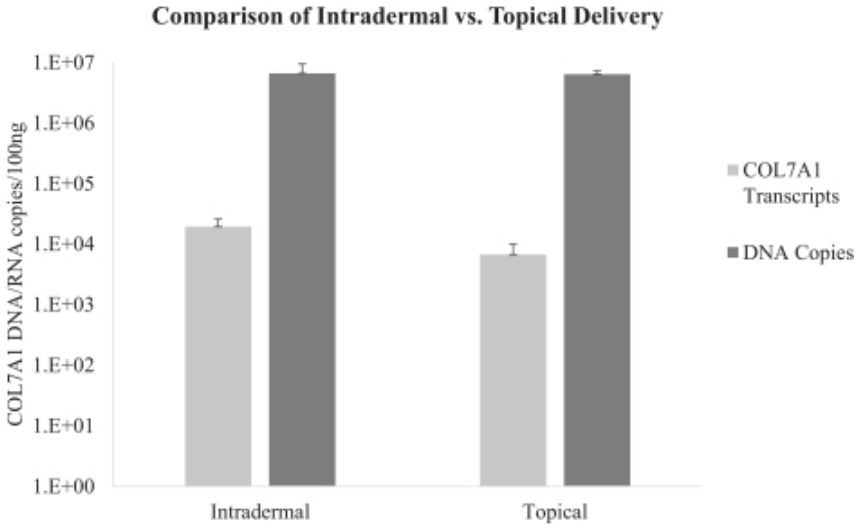

Figure 11. Comparison of Intradermal vs. Topical Delivery

We believe that collectively these studies suggest robust expression of functional COL7 in in-vitro and in-vivo models. Published studies have shown that, in animal models, expression of COL7 results in disease

correction. In addition, recent publications from Stanford University on an autologous approach have shown strong correlation between COL7 expression in animal studies and efficacy in Phase 1 human trials. Consequently, we intend to complete the list of preclinical studies as requested by the FDA and file an IND application for KB103 with the FDA in January 2018.

Clinical Development Plan for KB-103

In October 2016, we had a pre-IND meeting with the FDA. Based on responses from the FDA, we plan to submit an IND to initiate a Phase 1/2 clinical trial of KB103 in the first quarter 2018. To date, we have reviewed preclinical results using an intradermal formulation of KB103 with FDA. Subsequent to our FDA meeting we completed preclinical studies indicating a topical formulation of KB103 showed equivalent levels of COL7 expression as the intradermal formulation. Therefore, we plan to use the topical formulation in our Phase 1/2 study. We plan to study three adult patients with RDEB in the initial stage of the Phase 1/2 trial and upon seeing a prospect of benefit, we will seek to enroll six RDEB patients who are five years and older. The Phase 1/2 trial will evaluate evidence of COL7 expression and the presence of mature anchoring fibrils as measured through skin biopsy. Based on our pre-IND discussions with the FDA to date, we believe that expression of COL7 resulting from the application of KB103 will be sufficient to define clinical benefit. Results of this trial, which we expect to receive in mid-2018, will guide us in finalizing the design of a pivotal Phase 3 clinical trial. In this planned pivotal Phase 3 trial, up to 10 patients will be enrolled and evaluated. We anticipate enrolling both RDEB and DDEB patients in this trial. If successful, we believe the results of this Phase 3 trial could support submission of a Biologics License Application, or BLA, to the FDA in the United States and a Marketing Authorization Application, or MAA, to the EMA in Europe for our KB103 product candidate for the treatment of DEB. 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.

Future Opportunities

We believe our focus on the unique properties of dermatological diseases provides efficiencies as we select and pursue additional diseases associated with the skin. In addition, we will apply learnings from our near-term programs to future opportunities, which we believe will allow for rapid understanding and efficient drug development. We believe this will enable us to not only apply our gene therapy technology and specifically the STAR-D platform, across multiple severe, genetic diseases today, but across many broader indications associated with the skin, such as psoriasis and chronic wound healing.

We are starting to conduct preclinical studies on our second pipeline compound, KB104, to treat Netherton Syndrome, a severe autosomal recessive form of ichthyosis, associated with mutations in the SPINK5 gene and characterized by chronic skin inflammation, itch, dehydration and stunted growth. We intend to file an IND on KB104 in the third quarter of 2018 and begin clinical studies in the first quarter of 2019. We have also commenced research activities on ichthyosis vulgaris (also known as “ichthyosis simplex”), an inherited disease associated with mutation in the filaggrin gene. Ichthyosis vulgaris is the most common form of ichthyosis affecting around one in 250 people worldwide. We intend to start research activities on treatments for psoriasis, atopic dermatitis and chronic wounds in the fourth quarter of 2017.

Given the low prevalence of the rare or orphan diseases we are seeking to treat initially, we intend to internally establish a targeted commercial infrastructure that will be able to serve these patient population markets effectively. As we expand our portfolio to treat other non-orphan dermatological diseases, we intend to establish collaborative commercial alliances with other companies who are presently commercializing treatments for dermatological indications.

Following successful completion of a Phase 1 clinical trial of KB103, we intend to design, build and validate a commercial-scale cGMP facility for the upstream and downstream manufacturing processes of products based on our STAR-D platform.

Manufacturing

Our proprietary manufacturing process for clinical grade KB103 was developed and optimized internally based on our STAR-D platform, and involves both an upstream production process and downstream purification step. Recombinant viral vectors are made safe by removal of most of the viral machinery, including packaging proteins, so that they are incapable of or attenuated for replicating in human cells. However, in order to produce the recombinant virus, these viral proteins have to be re-introduced to the process so that the viral vector can be packaged. In most other viral vector production systems, the missing viral proteins are supplied in one or more individual helper plasmids, along with the base viral vector plasmid. All the plasmids are co-transfected into a production cell line in the presence of a transfection agent to facilitate viral vector production and packaging. The difficulty of this approach is that it requires cGMP-scale manufacturing and qualification of each of the packaging plasmids and optimization of the transfection method. Even with optimized reagents and methods, significant batch-to-batch variability is seen in viral vector yield and titer that, we believe, drives up the cost of viral vector manufacturing and scale-up, and increases the risk of failure during manufacturing.

Our proprietary upstream process for HSV-1 production avoids the aforementioned issues with AAV production systems. Our process requires three critical components:

- Production of a master virus seed stock, or MVSS;
- Production of complementing master cell bank, or MCB; and
- Optimized transduction parameters

The MVSS is scaled up from a single purified clone of the HSV-1 vector expressing the therapeutic COL7A1 gene. The MCB is a complementing cell line that stably expresses the HSV-1 viral proteins that are required for HSV-1 growth and packaging and have been deleted from the recombinant HSV-1 backbone. By introducing the deleted proteins into the MCB, as opposed to including them in the viral replication process via co-transfection of individual plasmids, we eliminate the need for multiple cGMP qualifications of the plasmids or variability in transfection efficiency from batch to batch, that other production processes face. Infection of the MCB with the MVSS at the optimal concentration results in production of the viral particle. Once the MCB and MVSS and the conditions of infection are established, virus production and resultant yield and titer are highly reproducible and scalable over multiple runs and the risk of failure is minimal.

Optimization of MCB, MVSS and production methods requires extensive knowledge and technical experience with the HSV-1 genome and significant upfront effort to design and select the best virus seed stock and complementing cell line. To date we have screened through hundreds of cell line clones to find the best complementing cell lines, and similarly designed and generated the optimal virus seed stocks for our portfolio candidates. The viral seed stock expresses the therapeutic proteins under the control of strong constitutive or tissue-specific promoters, and additional non-coding regulatory sequences have been included to optimize gene expression. We have also optimized the transduction conditions to reproducibly obtain high yields of the virus.

Unlike the upstream process, steps used to purify and concentrate the viral vector product are often common across different viral vector platforms, and usually involve multiple stages of clarification, concentration, and diafiltration with the ultimate goal to remove contaminants and concentrate the product. We believe that we have successfully developed a robust and reproducible process for purifying our viral vector to required concentrations for clinical use, while successfully removing contaminants to meet FDA guidelines.

We believe that the MVSS and MCB are a vital part of the production of KB103, as they will ensure the reproducible production of multiple clinical batches in a short six week cycle time frame and in a cost-effective manner.

We have made significant investments in developing the most comprehensive and optimized manufacturing process for our vector product candidate including:

- sufficient scale to support stability of KB103 with sufficient longevity that a small number of initial batches will likely provide adequate clinical supply up to pivotal Phase 3 trials;
- a proprietary vector manufacturing technique that produces a highly purified KB103;
- approximately 10 assays to accurately characterize our process and the HSV-1 vectors we produce; and
- a series of high-efficiency purification processes, which can be adapted and customized for our HSV-1 platform products.

We believe these improvements and our continued investment in our STAR-D platform will enable us to develop best in class, next generation gene therapy products for dermatological indications.

Our entire manufacturing process has been successfully transferred to a contract manufacturing organization, and cGMP manufacturing for our lead candidate, KB103, is scheduled for later this year. However, our long-term strategy is to bring our cGMP manufacturing in-house in order to maximally control our intellectual property.

Competition

The biotechnology and pharmaceutical industries are highly competitive. In particular, the field of gene therapy is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. Some of our competitors have substantially greater financial resources and larger research and development organizations. In addition, our experience in clinical trials, obtaining FDA and other regulatory approvals, manufacturing and commercialization of products may be more limited. At this time, there is no FDA- or EMA-approved treatment for EB. However, a number of companies are developing drug candidates for EB. At this time, there is no FDA- or EMA-approved treatment for DEB. However, with respect to companies and institutions developing a treatment for DEB we do not believe that we have any direct competitors who provide an allogeneic approach to treating this disease in the way we seek. As a result, we believe our competitors are more indirect and general in nature and fall into three broad categories:

- **Autologous Approaches:** We are aware of less than five companies and research institutions including Abeona, Fibrocell and Kings College who are developing autologous or grafting gene therapy approaches to treating DEB.
- **Palliative Treatments:** We are aware of companies such as Amicus Therapeutics who are developing product candidates taking a palliative approach to treating the disease.
- **Non-Gene Therapy:** We are aware that ProQR Therapeutics has a product candidate in preclinical development that intends to treat some forms of RDEB with a topical RNA-based treatment.

We believe that the major drawbacks of autologous therapies are the need for highly trained dermatologists, high cost of treatment and the need for sophisticated equipment setup and possible hospitalization. Additionally, because autologous treatments require processing patient’s own cells and tissues that takes time, there is a significant delay of eight months to more than one year between diagnosis and commencement of patient’s treatment. Potential palliative approaches may help mitigate some of the symptoms of the disease but do not treat the underlying disease at a molecular level. We expect that ProQR’s preclinical candidate will be able to treat only a subset of the many genetic defects that cause RDEB. Even if our competitors are able to successfully develop approved products for DEB, we believe there will remain a need for an off-the-shelf treatment such as KB103.

Intellectual Property

Protection of our intellectual property is an important part of our business. We seek patent protection in the United States and in other countries for our inventions and discoveries, and we develop and protect our key know-how and trade secrets relating to our platform technology and the products we are developing using our platform.

We have adopted a strategy of seeking patent protection in the United States and in other jurisdictions globally that we deem appropriate with respect to certain of our technologies relating to our products and process. As of June 9, 2017, we are actively prosecuting a patent application in front of the USPTO directed to our products and processes related to the treatment of epidermolysis bullosa, and a corresponding international patent application has been filed in accordance with the Paris Cooperation Treaty. Additionally, a patent application has been filed with the USPTO seeking protection for our core viral platform technologies, including the STAR-D platform. We continue to actively develop our portfolio through the filing of new patent applications, divisionals and continuations relating to our technologies as we deem appropriate.

In addition to patents and licenses, we rely on trade secrets and know-how to develop and maintain our competitive position. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, and obtain and maintain ownership of certain technologies, in part, through confidentiality agreements and invention assignment agreements with our employees, consultants and commercial partners. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how, as well as that of our licensees, including by implementing measures intended to maintain the physical security of research facilities and the physical and electronic security of our information technology systems.

Government Regulation and Product Approval

In the United States, the FDA regulates biologic products including gene therapy products under the Federal Food, Drug, and Cosmetic Act, or the FDCA, the Public Health Service Act, or the PHSA, and regulations and guidance implementing these laws. The FDCA, PHSA and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biologic products. Applications to the FDA are required before conducting human clinical testing of biologic products. Additionally, each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA, and in limited instances the National Institutes of Health, or the NIH, through its Recombinant DNA Advisory Committee, or RAC. FDA approval also must be obtained before marketing of biologic products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals to successfully develop and commercialize our product candidates.

Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Cellular, Tissue and Gene Therapies, or the OCTGT, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee, or the CTGTAC, to advise CBER on its reviews. CBER works closely with the NIH and the RAC, which makes recommendations to the NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. Although the FDA has not yet approved any human gene therapy product for sale, it has provided guidance for the development of gene therapy products. This guidance includes a growing body of guidance documents on chemistry, manufacturing and control, or CMC, clinical investigations and other areas of gene therapy development, all of which are intended to facilitate the industry’s development of gene therapy products.

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

U.S. Biologic Products Development Process

The FDA must approve a product candidate before it may be legally marketed in the United States. The process required by the FDA before a biologic product candidate may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests and in vivo studies in accordance with the FDA's current Good Laboratory Practice, or GLP, regulations and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an application for an IND exemption, which allows human clinical trials to begin unless FDA objects within 30 days;
- approval by an independent institutional review board, or IRB, reviewing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials according to the FDA's GCP regulations, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biologic product candidate for its intended use;
- preparation and submission to the FDA of a biologics license application, or BLA, for marketing approval that includes substantial evidence of safety, purity and potency from results of nonclinical testing and clinical trials;
- review of the product by an FDA advisory committee, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biologic product candidate is produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the biologic product candidate's identity, safety, strength, quality, potency and purity;
- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the BLA; and
- payment of user fees and FDA review and approval, or licensure, of the BLA.

Before testing any biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as in vivo studies to assess the potential safety and activity of the product candidate and to establish a rationale for therapeutic use. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs.

Concurrent with clinical trials, companies usually must complete some long-term preclinical testing, such as animal studies of reproductive adverse events and carcinogenicity, and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the drug in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate

and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf life.

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of an IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. With gene therapy protocols, if the FDA allows the IND to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA also may impose clinical holds on a biologic product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

Human Clinical Trials Under an IND

Clinical trials involve the administration of the biologic product candidate to healthy volunteers or patients under the supervision of qualified investigators which generally are physicians not employed by, or under, the control of the trial sponsor. Clinical trials are conducted under written study protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to a proposed clinical trial and places the trial on clinical hold, including concerns that human research subjects will be exposed to unreasonable health risks. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Accordingly, submission of an IND may or may not result in the FDA allowing clinical trials to commence. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers items such as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject, or their legal representative, reviews and approves the study protocol, and must monitor the clinical trial until completed. Clinical trials involving recombinant DNA also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research that utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials typically are conducted in three sequential phases that may overlap or be combined:

- Phase 1. The biologic product candidate initially is introduced into a small number of healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion and, if possible, to gain an early understanding of its effectiveness. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is

often conducted in patients. Phase 1 clinical trials of gene therapies are typically conducted in patients rather than healthy volunteers.

- Phase 2. The biologic product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product candidate for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- Phase 3. Phase 3 clinical trials are commonly referred to as "pivotal" studies, which typically denotes a study which presents the data that the FDA or other relevant regulatory agency will use to determine whether or not to approve a biologic product. In Phase 3 studies, the biologic product candidate is administered to an expanded patient population, generally at multiple geographically dispersed clinical trial sites in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling.
- Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA.

Additional Regulation for Gene Therapy Clinical Trials

In addition to the regulations discussed above, there are a number of additional standards that apply to clinical trials involving the use of gene therapy. The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene therapy trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these trials.

U.S. Review and Approval Processes

The results of the preclinical tests and clinical trials, together with detailed information relating to the product's CMC and proposed labeling, among other things, are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. For gene therapies, selecting patients with applicable genetic defects is a necessary condition to effective treatment. For the therapy we are currently developing, we believe that diagnoses based on existing genetic tests developed and administered by laboratories certified under the Clinical Laboratory Improvement Amendments, or CLIA, are sufficient to select appropriate patients and will be permitted by the FDA. Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. The FDA adjusts the PDUFA user fees on an annual basis. The PDUFA also imposes an annual product fee for biologics and an annual establishment license fee on facilities used to manufacture prescription biologics. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on

BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

The FDA reviews a BLA within 60 days of submission to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In that event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth, substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product candidate is safe and potent, or effective, for its intended use, has an acceptable purity profile and whether the product candidate is being manufactured in accordance with cGMP to assure and preserve the product candidate's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the product approval process, the FDA also will determine whether a REMS is necessary to assure the safe use of the product candidate.

REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events, and whether the product is a new molecular entity. A REMS could include medication guides, physician communication plans and elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product candidate within required specifications. Additionally, before approving a BLA, the FDA typically will inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements.

On the basis of the BLA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the biologic product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter.

If a product candidate receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post-marketing clinical trials, sometimes referred to as Phase 4 clinical trials, designed to further assess a biologic product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The FDA has agreed to specified performance goals in the review of BLAs under the PDUFA. One such goal is to review standard BLAs in 10 months after the FDA accepts the BLA for filing, and priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its

PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biologic product available in the United States for treatment of the disease or condition will be recovered from sales of the product). Orphan product designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. Competitors, however, may receive approval of different products for the same indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan medicinal product status in the European Union has similar, but not identical, benefits.

Expedited Development and Review Programs

In addition, the FDA is authorized to expedite the review of BLAs in several ways.

Fast Track Program

Under the Fast Track program, the sponsor of a biologic product candidate may request the FDA to designate the product for a specific indication as a Fast Track product concurrent with or after the filing of the IND. Biologic products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track BLA before the application is complete, a process known as rolling review. Any product submitted to FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as breakthrough therapy designation, priority review and accelerated approval.

Breakthrough Therapy Designation

To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives intensive guidance on an efficient drug development program; intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review; and rolling review.

Accelerated Approval

Drug or biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well-controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, FDA currently requires as a condition for accelerated approval pre-approval of promotional materials.

Fast Track designation, breakthrough therapy designation and accelerated approval do not change the standards for approval but may expedite the development or approval process.

Rare Pediatric Disease Priority Review Voucher

The FDA also offers a rare pediatric disease drug designation. If a drug receives the designation of a “rare pediatric disease” drug, it is eligible during the FDA marketing process to apply for a Rare Pediatric Disease Priority Review Voucher. According to the FDA website, under the Rare Pediatric Priority Review Voucher Program, 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.

Post-Approval Requirements

Rigorous and extensive FDA regulation of biologic products continues after approval, particularly with respect to cGMP requirements. Manufacturers are required to comply with applicable requirements in the cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biologic products include reporting of cGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product; recordkeeping requirements; reporting of adverse effects; reporting updated safety and efficacy information; and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA, together with a release protocol, showing a summary of the history of manufacture of the lot and the results of all tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency and effectiveness of biologic products. A sponsor also must comply with the FDA’s advertising and promotion requirements, such as the prohibition on promoting products for uses or in patient populations that are not described in the product’s approved labeling (known as “off-label use”).

Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of FDA approval of product candidates, some of a sponsor's U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period generally is one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biologic product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. Moreover, a given patent may only be extended once based on a single product. The USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Government Regulation Outside of the United States

In addition to regulations in the United States, sponsors are subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of biologic products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not a sponsor obtains FDA approval for a product, a sponsor must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application, much like the IND, prior to the commencement of human clinical trials. In the European Union, for example, a request for a Clinical Trial Authorization, or CTA, must be submitted to the competent regulatory authorities and the competent Ethics Committees in the European Union Member States in which the clinical trial takes place, much like FDA and the IRB, respectively. Once the CTA request is approved in accordance with the European Union and the European Union Member State's requirements, clinical trial development may proceed. The requirements and processes governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCPs and the applicable regulatory requirements of the country or countries in which the clinical trial is performed, as well as the ethical principles that have their origin in the Declaration of Helsinki (whichever provides the greater protection to the clinical trial participants).

Failure to comply with applicable foreign regulatory requirements may result in, among other things, fines; suspension, variation or withdrawal of regulatory approvals; product recalls; seizure of products; operating restrictions; and criminal prosecution.

Other Healthcare Laws and Regulations

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and use of pharmaceutical products that are granted marketing approval. Arrangements with third-party payors, existing or potential customers and referral sources are subject to broadly applicable fraud and abuse and other healthcare laws and regulations, and these laws and regulations may constrain the business or financial arrangements and relationships through which manufacturers market, sell and distribute the products for which they obtain marketing approval. Such restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in cash or kind, in exchange for, or to induce, either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under

federal healthcare programs 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 Patient Protection and Affordable Care Act, or 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 in order to commit a violation;

- the federal false claims and civil monetary penalties laws, including the civil False Claims Act, or the FCA, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government. Certain marketing practices, including off-label promotion, also may implicate the FCA. In addition, the PPACA codified case law that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA;
- the federal Physician Payments Sunshine Act, which requires 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 the CMS, information related to payments and other transfers of value to physicians, certain other healthcare providers and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and its implementing regulations, which imposes obligations, including mandatory contractual terms, with respect to safeguarding the transmission, security and privacy of protected health information;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; and
- state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; 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.

Violation of the laws described above or any other governmental laws and regulations may result in penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of operations, the exclusion from participation in federal and state healthcare programs, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and imprisonment. Furthermore, efforts to ensure that business activities and business arrangements comply with applicable healthcare laws and regulations can be costly.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any products for which we may obtain regulatory approval. In the United States, sales of any product candidates for which regulatory approval for commercial sale is obtained will depend in part on the availability of coverage and adequate reimbursement from third-party payors. Third-party payors include government authorities and health programs in the United States such as Medicare and Medicaid, managed care providers, private health insurers and other organizations. These third-party payors are increasingly reducing reimbursements for medical products and services. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of FDA-approved drugs for a particular indication. Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. European Union member states may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products, but monitor and control company profits. The downward pressure on health care costs has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations may not allow favorable reimbursement and pricing arrangements.

Health Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, for example, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected and continues to face major uncertainty due to the status of major legislative initiatives surrounding healthcare reform.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservation and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern the use, handling and disposal of various biologic, chemical and radioactive substances used in, and wastes generated by, operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. Equivalent laws have been adopted in other countries that impose similar obligations.

U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act, or FCPA, prohibits U.S. corporations and individuals from engaging in certain activities to obtain or retain business abroad or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. The scope of the FCPA includes interactions with certain healthcare professionals in many countries. Equivalent laws have been adopted in other foreign countries that impose similar obligations.

Employees

As of July 1, 2017, we had seven full-time employees, five of whom were primarily engaged in research and development activities and hold Masters or Ph.D., degrees in a scientific field. None of our employees is represented by a labor union and we consider our employee relations to be good.

Facilities

We lease approximately 5,065 square feet of combined laboratory and office space in Pittsburgh, Pennsylvania that we use in our research and development efforts. We established the geographic locations of our research and development operations based on proximity to the relevant market expertise and access to available talent pools. We presently plan on extending our current lease, which ends in October 2018.

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.

MANAGEMENT

Executive Officers and Directors

The following table provides information regarding our current executive officers and directors:

Name	Age	Position
Executive Officers		
Krish S. Krishnan	52	President and Chief Executive Officer, Chairman of the Board of Directors
Suma M. Krishnan	52	Founder, Chief Operating Officer and Director
Non-Employee Directors		
Daniel S. Janney(1)(2)	51	Director
Rockford Douglas Norby(1)(2)(3)	82	Director
Dino A. Rossi(1)(2)(3)	62	Director

(1) Member of the audit committee
(2) Member of the compensation committee
(3) Member of the nominating and corporate governance committee

Executive Officers

Krish S. Krishnan has served as our President and Chief Executive Officer and Chairman of our board of directors since our inception. Mr. Krishnan previously served as Chief Operating Officer of Intrexon Corporation (NYSE: XON) from 2011 to 2016, and as Chief Executive Officer and President of Pinnacle Pharmaceuticals, Inc. from 2009 to 2011. He also served as Chief Financial Officer and Chief Operating Officer of New River Pharmaceuticals, Inc. from 2004 to 2007 (previously listed on NASDAQ prior to its acquisition by Shire plc in 2007), and was a member of its board of directors from 2003 until 2007. He served as a Senior Managing Director of Third Security, LLC between 2001 and 2008 and as a board member of Biotie Therapies Oyj (BTH1V:Helsinki) between 2008 and 2009. He served as Managing Principal at Ariba before joining Third Security and also served with the management consulting firm A.T. Kearney, where he advised Fortune 50 companies on business strategy. Mr. Krishnan started his career as an engineer with E.I. Dupont de Nemours in Wilmington, Delaware. He received a B.S. in Mechanical Engineering from the Indian Institute of Technology, an M.S. in Engineering from the University of Toledo, and an M.B.A. in Finance from The Wharton School at the University of Pennsylvania.

Suma M. Krishnan is our founder and has served as our Chief Operating Officer and director since our inception. Ms. Krishnan has over two decades of experience in drug development. She previously served as Senior Vice President and head of the Human Therapeutics Division, as well as Senior Vice President of Regulatory Affairs at Intrexon Corporation (NYSE: XON) from 2012 to 2016. She previously served as Senior Vice President, Product Development at Pinnacle Pharmaceuticals, Inc. from 2009 to 2011. Ms. Krishnan served as Vice President, Product Development at New River Pharmaceuticals, Inc. from 2002 until 2007 (previously listed on NASDAQ prior to its acquisition by Shire plc in 2007). Prior to serving at New River Pharmaceuticals, Inc., Ms. Krishnan served in the following capacities: Director, Regulatory Affairs at Shire plc; Senior Project Manager at Pfizer, Inc. (NYSE: PFE), a multi-national pharmaceutical company; and a consultant at the Weinberg Group, a pharmaceutical and environmental consulting firm. Ms. Krishnan began her career as a discovery scientist for Janssen Pharmaceuticals, Inc., a subsidiary of Johnson & Johnson (NYSE: JNJ), in May 1991. Ms. Krishnan received an M.S. in Organic Chemistry from Villanova University, an M.B.A. from Institute of Management and Research (India) and an undergraduate degree in Organic Chemistry from Ferguson University (India).

Non-Employee Directors

Daniel S. Janney has served as a member of our board of directors since November 2016, and is chairman of the compensation committee and a member of the audit committee. Mr. Janney is a

Managing Director at Alta Partners, a life sciences venture capital firm, which he joined in 1996. Prior to joining Alta, from 1993 to 1996, he was a Vice President in Montgomery Securities' healthcare and biotechnology investment banking group, focusing on life sciences companies. Mr. Janney is a director of a number of companies including Esperion Therapeutics (NASDAQ:ESPR), Evolve Biosystems, Inc., Prolacta Bioscience, Inc., Sutro Biopharma and Viveve Medical, Inc. (NASDAQ:VIVE). He holds a Bachelor of Arts in History from Georgetown University and an M.B.A. from the Anderson School at the University of California, Los Angeles. We believe Mr. Janney's experience working with and serving on the boards of directors of life sciences companies and his experience working in the venture capital industry qualifies him to serve as a member of our Board.

R. Douglas Norby has served as a member of our board of directors since January 2017, and is chairman of the audit committee and a member of the compensation committee and the nominating and corporate governance committee. He also serves as lead independent director on the board of directors of Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN), where he has been a board member since September 1999. Mr. Norby has held positions of responsibility at many companies, including serving as Senior Vice President and Chief Financial Officer of Tessera, Inc., a provider of intellectual property for advanced semiconductor packaging; and as Senior Vice President and Chief Financial Officer of Zambeel, Inc., a data storage systems company. Mr. Norby has also served as Senior Vice President and Chief Financial Officer of Novalux, Inc., a manufacturer of lasers for optical networks; as Executive Vice President and Chief Financial Officer of LSI Logic Corporation, a semiconductor company which was acquired by Avago Technologies in 2013; as Senior Vice President and Chief Financial Officer of Mentor Graphics Corporation, a software company; and as President and Chief Operating Officer at Lucasfilm, Ltd., an entertainment company. His pharmaceutical experience includes serving as President of Pharmetrix Corporation, a drug delivery company, and as Senior Vice President and Chief Financial Officer of Syntex Corporation, a pharmaceutical company, which was later acquired by Roche Holding Ltd. Mr. Norby received a bachelor's degree in Economics from Harvard University and an MBA from Harvard Business School. We believe that Mr. Norby's extensive experience in the pharmaceutical industry as both an executive officer and a director qualifies him to serve as a member of our Board.

Dino A. Rossi has served as a member of our board of directors since June 2017, and is a member of the audit committee, compensation committee and the nominating and corporate governance committee. Mr. Rossi was previously employed by Balchem Corporation (NASDAQ: BCPC), where he served as Chief Executive Officer and President from October 1997 to April 2015, Chief Financial Officer from April 1996 to January 2004 and Treasurer from June 1996 to June 2003, as well as Executive Chairman from September 2015 to December 2016. He has also previously served as Vice President, Finance & Administration of Norit Americas Inc., a wholly owned subsidiary of Norit N.V. and Vice President, finance and Administration of Oakite Products Inc. He also previously served on the board of Scientific Learning Corp. Mr. Rossi holds a BS in Accounting from West Virginia University. We believe that Mr. Rossi's extensive leadership experience as an executive officer of a publicly traded company, as well as his financial expertise, qualifies him to serve as a member of our Board.

Board Composition

Board of Directors

Our board of directors may establish the authorized number of directors from time to time by resolution. Our board of directors currently consists of five members. The primary responsibilities of our board of directors are to provide oversight, strategic guidance, counseling and direction to our management. Our board of directors meets on a regular basis and additionally as required.

Classified Board of Directors

Our amended and restated certificate of incorporation and amended and restated bylaws that will be effective upon the closing of this offering provide for a classified board of directors consisting of three

classes of directors, each serving staggered three-year terms. As a result, one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Our directors will be divided among the three classes as follows.

- the Class I directors will be Ms. Krishnan and Mr. Norby, and their terms will expire at the first annual meeting of stockholders following this offering;
- the Class II directors will be Mr. Janney and Mr. Rossi, and their terms will expire at the second annual meeting of stockholders following this offering; and
- the Class III director will be Mr. Krishnan, and his term will expire at the third annual meeting of stockholders following this offering.

Each director's term continues until the election and qualification of his or her successor, or his or her earlier death, resignation or removal. Our amended and restated certificate of incorporation and amended and restated bylaws that will be effective upon the closing of this offering will authorize only our board of directors to fill vacancies on our board of directors. Any increase or decrease in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors.

The classification of our board of directors may have the effect of delaying or preventing changes in control of our company. See "Description of Capital Stock—Anti-Takeover Provisions—Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws Provisions."

Director Independence

Applicable NASDAQ rules require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, NASDAQ rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended, or the Exchange Act. The NASDAQ independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees and that neither the director nor any of his family members has engaged in various types of business dealings with us. In addition, under applicable NASDAQ rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Our board of directors has determined that all of our directors, except Krish S. Krishnan and Suma M. Krishnan are independent directors, as defined under applicable NASDAQ rules. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director and the transactions involving them described in the section entitled "Certain Relationships and Related-Party Transactions."

Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors or any other board committee: (i) accept, directly or indirectly, any consulting, advisory or other compensatory fee from the listed company or any of its subsidiaries; or (ii) be an affiliated person of the listed company or any of its subsidiaries.

Our executive officers are elected by, and serve at the discretion of, our board of directors. There are no family relationships among any of our non-executive directors and executive officers. With respect to executive officers, Mr. Krishnan and Ms. Krishnan are spouses and each serves on our board of directors.

Committees of Our Board of Directors

Our board of directors has an audit committee, a compensation committee and a nominating and corporate governance committee, each of which have the composition and responsibilities described below. Members serve on these committees until their resignation or until otherwise determined by our board of directors. Each committee operates under a charter approved by our board of directors. Following the closing of this offering, copies of each committee's charter will be posted on the investor relations section of our website at www.krystalbio.com.

Audit Committee

Our audit committee is composed of Messrs. Norby, Janney and Rossi. Mr. Norby is the chairperson of our audit committee. Messrs. Norby, Janney and Rossi each meet the requirements for independence under the current NASDAQ listing standards and SEC rules and regulations. Each member of our audit committee is financially literate. In addition, our board of directors has determined that Mr. Norby is an "audit committee financial expert" as defined in Item 407(d)(5)(ii) of Regulation S-K promulgated under the Securities Act. This designation does not impose any duties, obligations or liabilities that are greater than are generally imposed on members of our audit committee and our board of directors. Our audit committee is responsible for, among other things:

- our accounting and financial reporting processes, including our financial statement audits and the integrity of our financial statements;
- our compliance with legal and regulatory requirements;
- reviewing and approving related person transactions;
- selecting and hiring our registered independent public accounting firm;
- the qualifications, independence and performance of our independent auditors; and
- the preparation of the audit committee report to be included in our annual proxy statement.

Compensation Committee

Our compensation committee is composed of Messrs. Norby, Janney and Rossi. Mr. Janney is the chairperson of our compensation committee. The composition of our compensation committee meets the requirements for independence under the current NASDAQ listing standards and SEC rules and regulations. Each member of this committee is: (i) an outside director, as defined pursuant to Section 162(m) of the Internal Revenue Code of 1986, as amended, or the Code; and (ii) a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act. Our compensation committee is responsible for, among other things:

- evaluating, recommending, approving and reviewing executive officer and director compensation arrangements, plans, policies and programs;
- administering our cash-based and equity-based compensation plans; and
- making recommendations to our board of directors regarding any other board of director responsibilities relating to executive compensation.

Nominating and Corporate Governance Committee

Our nominating and corporate governance committee is composed of Messrs. Norby and Rossi. Mr. Rossi is the chairperson of our nominating and corporate governance committee. The composition of our nominating and corporate governance committee meets the requirements for independence under the current NASDAQ listing standards and SEC rules and regulations. Our nominating and corporate governance committee is responsible for, among other things:

- identifying, considering and recommending candidates for membership on our board of directors;

- overseeing the process of evaluating the performance of our board of directors; and
- advising our board of directors on other corporate governance matters.

Compensation Committee Interlocks and Insider Participation

None of our executive officers has served as a member of our compensation committee. None of the current members of our compensation committee has ever been an executive officer or employee of ours. None of our executive officers currently serves, or has served during the last completed fiscal year, on the compensation committee or board of directors of any other entity that has one or more executive officers serving as a member of our board of directors or compensation committee.

Codes of Business Conduct and Ethics

Effective upon the closing of this offering, we will adopt a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors, which will be available on our website at www.krystalbio.com. The reference to our website address in this prospectus does not include or incorporate by reference the information on our website into this prospectus. The nominating and corporate governance committee of our board of directors will be responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. In addition, we intend to post on our website all disclosures that are required by law or the listing standards of the applicable stock exchange concerning any amendments to, or waivers from, any provision of the Code of Conduct, to the extent required by the applicable rules and exchange requirements.

Non-Employee Director Compensation

The following table presents the total compensation for each person who served as a non-employee member of our board of directors in the year ended December 31, 2016. Other than as set forth in the table, in the year ended December 31, 2016, we did not pay any fees to, make any equity awards or non-equity awards to or pay any other compensation to the non-employee members of our board of directors.

In the future, we intend to adopt a formal policy regarding the compensation of all of our non-employee directors.

Name	Fees Earned or Paid in Cash	Option Awards(1)	Total
Daniel S. Janney	$ —	8,421(2)	$93,220(3)
R. Douglas Norby	—	4,211(4)	46,615(4)
Dino A. Rossi	—	—	— (5)

(1) The amounts reported in the Option Awards column represent the grant date fair value of the stock options granted to the directors during the year ended December 31, 2016 as computed in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718. The assumptions used in calculating the grant date fair value of the stock options reported in the Option Awards column are set forth in Note 10 to our audited financial statements included in this prospectus. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by our directors from the options.

(2) Daniel S. Janney holds 8,421 options, issued on November 10, 2016 at an exercise price of $11.07 per share.

(3) The total excludes two convertible promissory notes in the aggregate amount of $500,000 issued to Alta Bioequities, L.P., an investment entity owned and controlled by Mr. Janney, in November 2016 and May 2017. The notes are due in May 2018 and bear interest at a rate of 6% per annum. The notes, as amended in July 2017, are automatically convertible into preferred stock upon our closing of a preferred stock financing of at least $5 million, or into common stock upon the closing of an initial public offering. The conversion price of the notes in the event of an initial public offering is $18.64 per

share. The notes and accrued interest will convert into approximately shares of our common stock upon the completion of this offering.

(4) R. Douglas Norby holds 4,211 options, issued on November 30, 2016 at an exercise price of $11.07 per share.

(5) The total excludes 4,211 options issued to Dino A. Rossi on June 1, 2017, at an exercise price of $39.57 per share. The total also excludes a convertible promissory note in the amount of $750,000 issued to Mr. Rossi in June 2016. The note is due in May 2018 and bears interest at a rate of 6% per annum. The note is convertible into preferred stock automatically upon our closing of a preferred stock financing of at least $5 million, or into common stock upon the closing of an initial public offering. The conversion price of the 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. Assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, the note will convert into approximately shares of common stock upon the closing of this offering.

EXECUTIVE COMPENSATION

Due to our limited operating history as described in the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations," until 2017, we did not have an executive compensation program and we did not pay any employee compensation or issue any stock-based compensation to any employee or consultant. During 2016, Krish S. Krishnan acted as our President and Chief Executive Officer and Suma M. Krishnan acted as our Chief Operating Officer. We refer to Mr. Krishnan and Ms. Krishnan as our named executive officers for 2016. Neither Mr. Krishnan nor Ms. Krishnan was an employee of the Company in 2016. In June 2017, we hired Ms. Krishnan as our Chief Operating Officer. In July 2017, we hired Mr. Krishnan as our President and Chief Executive Officer.

Summary Compensation Table

We did not pay Mr. Krishnan or Ms. Krishnan any base salary, bonus or stock-based or other compensation during 2016. See "Executive Compensation —Executive Employment Arrangements" for a description of the current and future compensation of Mr. Krishnan and Ms. Krishnan.

Executive Employment Arrangements

Krish S. Krishnan

We entered into an "at-will" employment agreement with Krish S. Krishnan dated as of July 1, 2017. Prior to entering into this agreement, between April 15, 2016, the date we commenced operations, and June 30, 2017, Mr. Krishnan served as our President and Chief Executive Officer without compensation. Under the terms of the employment agreement, Mr. Krishnan continues to serve as President and Chief Executive Officer with a base salary of sixty thousand dollars ($60,000) per year, in addition to benefits made available by the Company to similarly-situated employees. Mr. Krishnan's employment agreement provides that he will be bound by the terms of the Company's Proprietary Information and Inventions Agreement and that he shall not disclose to the Company any third party proprietary information or trade secrets.

Suma M. Krishnan

We entered into an "at-will" employment agreement with Suma M. Krishnan dated as of May 1, 2017. Prior to entering into this agreement, and between the dates of April 15, 2016, the date we commenced operations, and May 1, 2017, Ms. Krishnan served as the Company's Chief Operating Officer without compensation. Under the terms of the employment agreement, Ms. Krishnan continues to serve as the Chief Operating Officer. She receives a base salary of sixty thousand dollars ($60,000) per year, in addition to benefits made available by the Company to similarly-situated employees. Ms. Krishnan's employment agreement provides that she will be bound by the terms of the Company's Proprietary Information and Inventions Agreement and that she shall not disclose to the Company any third party proprietary information or trade secrets.

Pooja Agarwal

We entered into an "at-will" employment agreement with Pooja Agarwal dated as of May 1, 2017. Prior to entering into this agreement, and between the dates of May 1, 2016 and May 1, 2017, Ms. Agarwal served as our consultant. Under the terms of the employment agreement, Ms. Agarwal serves as the Vice President of Product Development with a base salary of one hundred and sixty-five thousand dollars ($165,000) per year, in addition to benefits made available by the Company to similarly-situated employees. Ms. Agarwal's employment agreement provides that she will be bound by the terms of the Company's Proprietary Information and Inventions Agreement and that she shall not disclose to the Company any third-party proprietary information or trade secrets.

Equity Incentive Plan

Krystal Biotech, LLC 2016 Equity Incentive Plan

The Krystal Biotech, LLC 2016 Equity Incentive Plan, or the 2016 Plan, was adopted on October 1, 2016 for the purpose of offering selected persons a proprietary interest in our success, or increasing such interest, by the grant of awards. The 2016 Plan terminated upon our conversion into a Delaware corporation on March 31, 2017, and options to purchase incentive units issued under that plan were, in accordance with the terms of the conversion and the original option awards, automatically converted into options to purchase shares of our common stock at the same exercise price per share.

Under the terms of the 2016 Plan as it was in effect, our Board of Managers was authorized to issue options to purchase up to 42,105 incentive units. The number and kind of incentive units outstanding, and made available for future grants, would have been automatically adjusted in the event of any subdivision or combination of outstanding units, a recapitalization, a spin-off, a reclassification, a merger or consolidation or any other similar occurrence. The original term of the 2016 Plan expired on September 30, 2026.

Administration

The 2016 Plan was administered by our Board of Managers, which had full authority and discretion to take any actions it deems necessary or advisable for the plan's administration. All decisions, interpretations, and other actions of the Board of Managers were final and binding on all plan participants.

Grants of Awards and Stock Options

Only service providers were eligible for the grant of incentive units, including incentive units to be issued on exercise of options, under the 2016 Plan. All awards under the plan were subject to any special forfeiture conditions, rights of repurchase, rights of first refusal, and other transfer restrictions determined by the Board of Managers in its discretion. The Board of Managers also had the sole discretion to decide the vesting schedules of awards.

Award Exercise or Purchase Price

The per incentive unit exercise price for an option, was determined by the Board of Managers in its sole discretion, and was required to equal or exceed one hundred percent (100%) of the fair market value per incentive unit on the date of grant. Any option granted under the 2016 Plan was exercisable at such times and under such conditions as determined by the Board of Managers, in accordance with the terms of the plan and the award agreement.

Corporate Transaction and Change in Control Provisions

The 2016 Plan granted us the ability to determine the treatment of outstanding options to purchase incentive units upon the occurrence of any "company event," which included a sale of all or substantially all of the Company's assets, an acquisition of at least fifty percent (50%) of the outstanding units of the Company, the liquidation or dissolution of the Company, or a similar transaction, subject to the terms of any option agreement requiring specific treatment in such circumstances. Option award agreements issued under the 2016 Plan provided that options granted could be exercised, even if vested, only on the earlier of a company event or an incorporation.

Krystal Biotech, Inc. 2017 Stock Incentive Plan

The Krystal Biotech, Inc. 2017 Stock Incentive Plan, or the 2017 Plan, became effective on March 31, 2017 and was approved by shareholders on March 31, 2017. The 2017 Plan will continue in effect for a term of ten (10) years unless sooner terminated. The purpose of the plan is to attract and retain the best available personnel, to provide additional incentives to employees, directors, and consultants, and to promote the success of the Company's business. Subject to adjustment as discussed below, the 2017 Plan

authorizes up to 42,900 shares of Company common stock for issuance pursuant to the terms of the plan. If and to the extent that awards are forfeited, canceled, or expire, the shares subject to the award will again be available for grant under the 2017 Plan. Additionally, to the extent any shares covered by an award are surrendered or withheld in payment of any exercise or purchase price, or in satisfaction of tax withholding obligations associated with the award, such shares subject to the award will again be available for grant under the 2017 Plan.

In the case of any increase or decrease in the number of issued shares due to a stock split, reverse stock split, stock dividend, recapitalization, or other such transaction affecting shares, or, in the case of a corporate merger, consolidation, acquisition, reorganization, or liquidation, then proportionate adjustments shall be made for: (i) the number of shares covered by each outstanding award; (ii) the number of shares authorized for issuance but as to which no awards have been granted (or which have been returned); (iii) the exercise or purchase price of each outstanding award; the maximum number of shares which may be granted to any grantee; and (iv) any other terms that the 2017 Plan administrator determines.

Administration

The 2017 Plan is administered by our board of directors or any committees designated by the board of directors to administer the plan. The administrator has the authority, in its discretion, to select the employees, directors and consultants to whom awards may be granted and to determine whether and to what extent such awards will be granted. Subject to the terms of the 2017 Plan, the administrator shall determine the provisions, terms and conditions of each award including, but not limited to, the award vesting schedule, repurchase provisions, rights of first refusal, forfeiture provisions, form of payment upon settlement of the award, payment contingencies, the number of shares or amount of other consideration to be covered by awards and satisfaction of any performance criteria (which the administrator will establish). Our board of directors may also authorize officers to grant awards, subject to limitations under applicable law.

Grants of Awards and Stock Options

Awards granted under the 2017 Plan may include, without limitation, options (including incentive stock options or non-qualified stock options), stock appreciation rights (SARs), dividend equivalent rights, restricted stock, and restricted stock units. Awards other than incentive stock options may be granted to employees, directors, and consultants, whereas incentive stock options may be granted only to employees of the Company or its parent or subsidiary. The term of each award may not exceed ten (10) years from its date of grant (or five (5) years, in the case of an incentive stock option granted to a ten percent (10%) shareholder within the meaning of Section 422(b)(6) of the Internal Revenue Code).

Award Exercise or Purchase Price

Determination of the exercise or purchase price of any of the awards issued under the 2017 Plan is as follows:

- the per share exercise price of incentive stock options must equal or exceed one hundred percent (100%) of the fair market value per share on the date of grant (unless granted to an employee who, at the time of such grant is a ten percent (10%) shareholder within the meaning of Section 422(b)(6) of the Internal Revenue Code, in which case the exercise price must exceed one hundred and ten percent (110%) of the fair market value per share on the date of grant);
- the base appreciation amount of SARs must equal or exceed one hundred percent (100%) of the fair market value per share on the date of grant;
- the per share exercise or purchase price, if any, of performance-based compensation awards must equal or exceed one hundred percent (100%) of the fair market value per share on the date of grant; and

- the per share exercise price of non-qualified stock options, the per share purchase price for the sale of shares, and the per share price of all other awards shall be determined by the 2017 Plan administrator.

Corporate Transaction and Change in Control Provisions

Under the 2017 Plan, a corporate transaction includes: (i) a merger or consolidation in which the Company is not the surviving entity or certain reverse mergers in which the Company is the surviving entity but all outstanding common stock is converted or more than fifty percent (50%) of the total combined voting power of the Company's outstanding securities are transferred to a new shareholder(s); (ii) the sale of all or substantially all of the Company's assets; the complete liquidation or dissolution of the Company; or (iii) an acquisition of securities possessing more than fifty percent (50%) of the total combined voting power of the Company's outstanding securities. Under the 2017 Plan, a change in control is defined as either the acquisition of more than fifty percent (50%) of the total combined voting power of the Company's outstanding securities pursuant to a hostile tender or exchange offer; or a change in the composition of the board of directors taking place over one year or less, whereby a majority of Board members cease to be continuing directors by reason of contested elections for Board membership.

Effective upon the consummation of a corporate transaction, all outstanding awards under the 2017 Plan shall terminate, unless they are assumed. The plan administrator shall have the authority, exercisable either in advance or at the time of any actual or anticipated corporate transaction or change in control, to accelerate vesting and exercisability of outstanding unvested awards, as well as the release from restrictions on transfer and repurchase or forfeiture rights of such awards on such terms and conditions as the administrator may specify, including conditioning such acceleration upon the subsequent termination of the employment of the grantee within a specified period following the effective date of the corporate transaction or change in control.

Section 162(m)

The 2017 Plan is structured so that stock options and other performance-based awards may qualify for an exemption to the deduction limitation contained in Section 162(m) of the Internal Revenue Code, to the extent applicable.

Limitation on Liability and Indemnification Matters

Our restated certificate of incorporation that will become effective in connection with the completion of this offering contains provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by the Delaware General Corporation Law, or DGCL. Consequently, our directors will not be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the DGCL; or
- any transaction from which the director derived an improper personal benefit.

Our restated certificate of incorporation and our restated bylaws that will become effective in connection with the completion of this offering require us to indemnify our directors and officers to the maximum extent not prohibited by the DGCL and allow us to indemnify other employees and agents as set forth in the DGCL. Subject to certain limitations, our restated bylaws also require us to advance expenses incurred by our directors and officers for the defense of any action for which indemnification is required or permitted.

We have entered, and intend to continue to enter, into separate indemnification agreements with our directors, officers and certain of our key employees, in addition to the indemnification provided for in our restated certificate of incorporation and restated bylaws. These agreements, among other things, require us to indemnify our directors, officers and key employees for certain expenses, including attorneys' fees, judgments, penalties, fines and settlement amounts actually incurred by these individuals in any action or proceeding arising out of their service to us or any of our subsidiaries or any other company or enterprise to which these individuals provide services at our request. Subject to certain limitations, our indemnification agreements also require us to advance expenses incurred by our directors, officers and key employees for the defense of any action for which indemnification is required or permitted.

We believe that provisions of our restated certificate of incorporation, bylaws and indemnification agreements are necessary to attract and retain qualified directors, officers and key employees. We also maintain directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our restated certificate of incorporation and restated bylaws may discourage stockholders from bringing a lawsuit against our directors and officers for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions.

At present, there is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, executive officers or persons controlling us, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

CERTAIN RELATIONSHIPS AND RELATED PARTY AND OTHER TRANSACTIONS

In addition to the executive officer and director compensation arrangements discussed above under "Management—Non-Employee Director Compensation" and "Executive Compensation," below we describe transactions since our inception to which we have been or will be a participant, in which the amount involved in the transaction exceeds or will exceed $120,000 and in which any of our directors, executive officers or beneficial holders of more than 5% of any class of our capital stock, or any immediate family member of, or person sharing the household with, any of these individuals, had or will have a direct or indirect material interest.

Equity Financings

Seed Financing

On April 15, 2016, we authorized and issued 100 member units in the aggregate to Krish S. Krishnan and Suma M. Krishnan for aggregate proceeds of $100 thousand. On September 30, 2016, we converted all of the member units into an aggregate of 2,838 preferred units at an issue price of $35.26 per unit plus 775,752 common units, and issued an additional 21,410 preferred units to Mr. Krishnan and Ms. Krishnan at the same price for aggregate proceeds of $754 thousand. On December 27, 2016, we issued an additional 15,666 preferred units in the aggregate to Mr. Krishnan and Ms. Krishnan at the same price for aggregate proceeds of $552 thousand.

We converted from a California limited liability company to a Delaware corporation on March 31, 2017, and upon such entity conversion, all preferred units were converted to preferred stock on a 1:1 basis and all common units were converted to common stock on a 1:1 basis. Each share of preferred stock will automatically convert into one share of our common stock immediately prior to the completion of this offering.

The following table summarizes the preferred stock and common stock purchased by our directors, executive officers and beneficial holders of more than 5% of our capital stock:

Name of Stockholder	Shares of Preferred Stock	Total Purchase Price
Krish S. Krishnan	19,957	$ 703,208
Suma M. Krishnan	19,957	703,208

Convertible Notes Financing

In November 2016 and May 2017, we issued two convertible promissory notes in the aggregate amount of $500,000 to Alta Bioequities, L.P., an investment entity owned and controlled by a member of our board of directors, Daniel S. Janney. The notes are due in May 2018 and bear interest at a rate of 6% per annum. The notes, as amended in July 2017, are convertible into preferred stock automatically upon our closing of a preferred stock financing of at least $5 million, or upon the closing of an initial public offering. The conversion price of the notes in the event of a preferred financing is the lower of 80% of the sales price of the preferred stock, or the price at which the preferred stock would have been issued had we been valued at $16 million at the time of the financing. The conversion price of the notes in the event of an initial public offering is $18.64 per share. The notes and accrued interest will convert into approximately shares of common stock upon the closing of this offering.

In December 2016, we issued a convertible promissory note in the aggregate amount of $448,000 to the Krishnan Family Trust, a trust controlled by our President, Chief Executive Officer and Chairman of the board of directors, Krish S. Krishnan, and our founder, Chief Operating Officer and director, Suma M. Krishnan. The note is due in June 2018 and bears interest at a rate of 6% per annum. The note, as amended in July 2017, is convertible into preferred stock automatically upon our closing of a preferred stock financing of at least $5 million, or upon the closing of an initial public offering. The conversion price of the note in the event of a preferred financing is the lower of 80% of the sales price of the preferred stock, or the price at which the preferred stock would have been issued had we been valued at $16 million

at the time of the financing. The conversion price of the note in the event of an initial public offering is $18.64 per share. The note and accrued interest will convert into approximately shares of common stock upon the closing of this offering.

In June 2017, we issued a convertible promissory note to Dino A. Rossi, a member of our board of directors, in the amount of $750,000. The note is due in May 2018 and bears interest at a rate of 6% per annum. The note is convertible into preferred stock automatically upon our closing of a preferred stock financing of at least $5 million, or into common stock upon the closing of an initial public offering. The conversion price of the 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. Assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, the note will convert into approximately shares of common stock upon the closing of this offering.

The following table summarizes the convertible promissory notes purchased by our directors, executive officers and beneficial holders of more than 5% of our capital stock.

Name of Noteholder	Total Purchase Price
Alta Bioequities, L.P.(1)	$ 500,000
Dino A. Rossi	750,000
Krishnan Family Trust(2)	448,000

(1) Represents an investment entity owned and controlled by Daniel S. Janney, a member of our board of directors.
(2) Represents a revocable trust owned and controlled by Krish S. Krishnan, our President, Chief Executive Officer and Chairman of our board of directors, and Suma M. Krishnan, our founder, Chief Operating Officer and director.

Equity Grants to Executive Officers and Directors

We have granted stock options to certain of our executive officers and certain directors, as more fully described in the sections entitled “Executive Compensation” and “Management—Non-Employee Director Compensation,” respectively.

Indemnification Agreements

In connection with this offering, we intend to enter into indemnification agreements with each of our directors and executive officers. The indemnification agreements, our restated certificate of incorporation and our restated bylaws will require us to indemnify our directors to the fullest extent not prohibited by Delaware law. Subject to certain limitations, our restated bylaws also require us to advance expenses incurred by our directors and officers. For more information regarding these agreements, see “Executive Compensation—Limitations on Liability and Indemnification Matters.”

Review, Approval or Ratification of Transactions with Related Parties

In connection with this offering, we adopted a written related-person transactions policy that provides that our executive officers, directors, nominees for election as a director, beneficial owners of more than 5% of our common stock and any members of the immediate family of the foregoing persons, are not permitted to enter into a material related-person transaction with us without the review and approval of our audit committee, or a committee composed solely of independent directors in the event it is inappropriate for our audit committee to review such transaction due to a conflict of interest. The policy provides that any request for us to enter into a transaction with an executive officer, director, nominee for election as a director, beneficial owner of more than 5% of our common stock or with any of their immediate family members or affiliates, in which the amount involved exceeds $120,000 will be presented to our audit committee for review, consideration and approval. In approving or rejecting any such proposal, we expect that our audit committee will consider the relevant facts and circumstances available and deemed relevant to the audit committee, including, but not limited to, whether the transaction is on terms no less favorable than terms generally available to an unaffiliated third party under the same or similar circumstances and the extent of the related person’s interest in the transaction.

PRINCIPAL STOCKHOLDERS

The following table sets forth information regarding beneficial ownership of our common stock as of July 1, 2017, as adjusted to reflect the shares of common stock to be issued and sold by us in this offering, by:

- each person or group of affiliated persons known by us to be the beneficial owner of more than 5% of our common stock;
- each of our named executive officers;
- each of our directors; and
- all of our executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC and the information is not necessarily indicative of beneficial ownership for any other purpose. Unless otherwise indicated below, to our knowledge, the persons and entities named in the table have sole voting and sole investment power with respect to all shares that they beneficially own, subject to community property laws where applicable. In computing the number of shares of our common stock beneficially owned by a person and the percentage ownership of that person, we deemed outstanding shares of our common stock subject to options or warrants held by that person that are currently exercisable or exercisable within 60 days of July 1, 2017. We did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person.

We have based percentage ownership of our common stock prior to this offering on 815,666 shares of our common stock outstanding as of July 1, 2017, including the conversion of our preferred stock immediately prior to this offering and excluding the conversion of any outstanding convertible promissory notes and accrued interest thereon. Percentage ownership of our common stock after this offering assumes the sale by us of shares of common stock in this offering and the conversion of our outstanding convertible promissory notes and accrued interest thereon.

Unless otherwise indicated, the address of each beneficial owner listed on the table below is c/o Krystal Biotech, Inc., 2100 Wharton Street, Suite 701, Pittsburgh, Pennsylvania 15203.

	Shares Beneficially Owned Prior to this Offering		Shares Beneficially Owned After this Offering	
Name of Beneficial Owner	Number	Percentage	Number	Percentage
Named Executive Officers and Directors				
Krish S. Krishnan	407,833	50%	(1)	%
Suma M. Krishnan	407,833	50	(1)	
Pooja Agarwal	—	—	—	—
Daniel S. Janney	—	—	(2)	
R. Douglas Norby	—	—	—	—
Dino A. Rossi	—	—	(3)	
5% Stockholders				
Krish S. Krishnan	407,833	50%	(1)	%
Suma M. Krishnan	407,833	50	(1)	

(1) Reflects conversion of $448,000 principal amount of convertible promissory notes, plus accrued interest at a rate of 6% per annum, into shares of common stock as of the closing of this offering.

(2) Reflects conversion of $500,000.00 principal amount of convertible promissory notes, plus accrued interest at a rate of 6% per annum, held by an investment entity owned and controlled by Mr. Janney, into shares of common stock as of the closing of this offering.

(3) Reflects conversion of $750,000.00 principal amount of convertible promissory notes, plus accrued interest at a rate of 6% per annum, into shares of common stock as of the closing of this offering.

DESCRIPTION OF CAPITAL STOCK

Upon the completion of this offering and the filing of our amended and restated certificate of incorporation, our authorized capital stock will consist of shares of common stock, $0.00001 par value per share. The following description summarizes the most important terms of our capital stock. Because it is only a summary, it does not contain all the information that may be important to you. For a complete description, you should refer to our amended and restated certificate of incorporation and amended and restated bylaws, which are included as exhibits to the registration statement of which this prospectus forms a part.

Pursuant to the provisions of our amended and restated certificate of incorporation and the terms of the preferred stock and note purchase agreements, as amended, all of our outstanding preferred stock and convertible promissory notes convertible into common stock will automatically convert into common stock effective immediately prior to the completion of this offering. Assuming the conversion of all outstanding shares of our preferred stock into 39,914 shares of common stock and all outstanding convertible promissory notes into shares of common stock, as of , 2017, there were shares of our common stock issued and outstanding, held by approximately stockholders of record, and no shares of our preferred stock outstanding.

Common Stock

Dividend Rights

The holders of our common stock are entitled to receive dividends out of funds legally available if our board of directors, in its discretion, determines to issue dividends and then only at the times and in the amounts that our board of directors may determine. See “Dividend Policy” above.

Voting Rights

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders. We have not provided for cumulative voting for the election of directors in our restated certificate of incorporation. Accordingly, holders of a majority of the shares of our common stock will be able to elect all of our directors. Our restated certificate of incorporation will establish a classified board of directors, divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms.

No Preemptive or Similar Rights

Our common stock is not entitled to preemptive rights, and is not subject to conversion, redemption or sinking fund provisions.

Right to Receive Liquidation Distributions

Upon our liquidation, dissolution or winding-up, the assets legally available for distribution to our stockholders would be distributable ratably among the holders of our common stock at that time, subject to prior satisfaction of all outstanding debt and liabilities.

Preferred Stock

Pursuant to the provisions of our amended and restated certificate of incorporation which will become effective in connection with the completion of this offering, each currently outstanding share of preferred stock will automatically be converted into one share of common stock effective immediately prior to the completion of this offering. Following this offering, no shares of preferred stock will be outstanding.

Pursuant to our amended and restated certificate of incorporation that will become effective in connection with the completion of this offering, our board of directors will be authorized, subject to

limitations prescribed by Delaware law, to issue from time to time up to shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each series and to fix the designation, powers, preferences and rights of the shares of each series and any of their qualifications, limitations or restrictions, in each case without further vote or action by our stockholders. Our board of directors will also be able to increase or decrease the number of shares of any series of preferred stock, but not below the number of shares of that series then outstanding, without any further vote or action by our stockholders. Our board of directors may be able to authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of our common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of our company and might adversely affect the market price of our common stock and the voting and other rights of the holders of our common stock. We have no current plan to issue any shares of preferred stock.

Stock Options

As of March 31, 2017, we had outstanding options to purchase an aggregate of 25,263 shares of our common stock, with an exercise price of $11.07 per share. Subsequent to March 31, 2017 and before June 30, 2017, we issued options to purchase an aggregate of 10,211 shares of our common stock, with a weighted average exercise price of $22.00 per share.

Convertible Promissory Notes

As of March 31, 2017, we had outstanding 11 convertible promissory notes in an aggregate amount of approximately $2.1 million, each due May 2018 at an interest rate of 6% per annum. These notes, as subsequently amended in July 2017, are convertible into preferred stock automatically upon our closing of a preferred stock financing of at least $5 million, or upon the closing of an initial public offering. The conversion price of the notes in the event of a preferred financing is the lower of 80% of the sales price of the preferred stock, or the price at which the preferred stock would have been issued had we been valued at $16 million at the time of the financing. The conversion price of the notes in the event of an initial public offering is $18.64 per share. The notes and accrued interest will convert into approximately shares of common stock upon the closing of this offering.

We issued an additional four notes for an aggregate principal amount of $1.25 million on identical terms after March 31, 2017. The conversion price of the notes in the event of an initial public offering is $18.64 per share. The notes and accrued interest will convert into approximately shares of common stock upon the closing of this offering. We also issued a note for $750,000 in June 2017, which is due in May 2018 and bears interest at a rate of 6% per annum. The June 2017 note is convertible into preferred stock automatically upon our closing of a preferred stock financing of at least $5 million, or into common stock upon the closing of an initial public offering. The conversion price of the 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. Assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, the notes will convert into approximately shares of common stock upon the closing of this offering.

Anti-Takeover Provisions.

The provisions of Delaware law, our restated certificate of incorporation and our restated bylaws, as we expect they will be in effect immediately prior to the completion of this offering, could have the effect of delaying, deferring or discouraging another person from acquiring control of our company. These provisions, which are summarized below, may have the effect of discouraging takeover bids. They are also designed, in part, to encourage persons seeking to acquire control of us to negotiate first with our board of directors. We believe that the benefits of increased protection of our potential ability to negotiate with an unfriendly or unsolicited acquirer outweigh the disadvantages of discouraging a proposal to acquire us because negotiation of these proposals could result in an improvement of their terms.

Delaware Law

We are subject to the provisions of Section 203 of the Delaware General Corporation Law, or DGCL, regulating corporate takeovers. In general, DGCL Section 203 prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder for a period of three years following the date on which the person became an interested stockholder unless:

- prior to the date of the transaction, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder: (i) shares owned by persons who are directors and also officers; and (ii) shares owned by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- at or subsequent to the date of the transaction, the business combination is approved by the board of directors of the corporation and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66.67% of the outstanding voting stock that is not owned by the interested stockholder.

Generally, a business combination includes a merger, asset or stock sale, or other transaction or series of transactions together resulting in a financial benefit to the interested stockholder. An interested stockholder is a person who, together with affiliates and associates, owns or, within three years prior to the determination of interested stockholder status, did own 15% or more of a corporation's outstanding voting stock. We expect the existence of this provision to have an anti-takeover effect with respect to transactions our board of directors does not approve in advance. We also anticipate that DGCL Section 203 may also discourage attempts that might result in a premium over the market price for the shares of common stock held by stockholders.

Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws Provisions

Our amended and restated certificate of incorporation and our amended and restated bylaws, as we expect they will be in effect immediately prior to the completion of this offering, include a number of provisions that could deter hostile takeovers or delay or prevent changes in control of our company, including the following:

- *Board of Directors Vacancies.* Our amended and restated certificate of incorporation and amended and restated bylaws will authorize only our board of directors to fill vacant directorships, including newly created seats. In addition, the number of directors constituting our board of directors will be permitted to be set only by a resolution adopted by a majority vote of our entire board of directors. These provisions would prevent a stockholder from increasing the size of our board of directors and then gaining control of our board of directors by filling the resulting vacancies with its own nominees. This makes it more difficult to change the composition of our board of directors but promotes continuity of management.
- *Classified Board.* Our amended and restated certificate of incorporation and amended and restated bylaws will provide that our board of directors will be classified into three classes of directors, each with staggered three-year terms. A third party may be discouraged from making a tender offer or otherwise attempting to obtain control of us as it is more difficult and time consuming for stockholders to replace a majority of the directors on a classified board of directors. See "Management—Board of Directors."
- *Stockholder Action; Special Meetings of Stockholders.* Our amended and restated certificate of incorporation will provide that our stockholders may not take action by written consent, but may

only take action at annual or special meetings of our stockholders. As a result, a holder controlling a majority of our capital stock would not be able to amend our restated bylaws or remove directors without holding a meeting of our stockholders called in accordance with our restated bylaws. Further, our restated bylaws and restated certificate of incorporation will provide that special meetings of our stockholders may be called only by a majority of our board of directors, the chairman of our board of directors, or our Chief Executive Officer, thus prohibiting a stockholder from calling a special meeting. These provisions might delay the ability of our stockholders to force consideration of a proposal or for stockholders controlling a majority of our capital stock to take any action, including the removal of directors.

- *Advance Notice Requirements for Stockholder Proposals and Director Nominations.* Our amended and restated bylaws will provide advance notice procedures for stockholders seeking to bring business before our annual meeting of stockholders or to nominate candidates for election as directors at our annual meeting of stockholders. Our restated bylaws also will specify certain requirements regarding the form and content of a stockholder's notice. These provisions might preclude our stockholders from bringing matters before our annual meeting of stockholders or from making nominations for directors at our annual meeting of stockholders if the proper procedures are not followed. We expect that these provisions might also discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company.
- *No Cumulative Voting.* The Delaware General Corporation Law provides that stockholders are not entitled to the right to cumulate votes in the election of directors unless a corporation's certificate of incorporation provides otherwise. Our amended and restated certificate of incorporation will not provide for cumulative voting.
- *Directors Removed Only for Cause.* Our amended and restated certificate of incorporation will provide that stockholders may remove directors only for cause and only by the affirmative vote of the holders of at least two-thirds of our outstanding common stock.
- *Amendment of Charter Provisions.* Any amendment of the above expected provisions in our restated certificate of incorporation would require approval by holders of at least two-thirds of our outstanding common stock.
- *Issuance of Undesignated Preferred Stock.* Our board of directors has the authority, without further action by the stockholders, to issue up to shares of undesignated preferred stock with rights and preferences, including voting rights, designated from time to time by our board of directors. The existence of authorized but unissued shares of preferred stock would enable our board of directors to render more difficult or to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or other means.
- *Choice of Forum.* Our amended and restated certificate of incorporation will provide that the Court of Chancery of the State of Delaware will be the exclusive forum for: any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our restated certificate of incorporation or our restated bylaws; any action to interpret, apply, enforce or determine the validity of our restated certificate of incorporation or our restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable.

Transfer Agent and Registrar

Upon the completion of this offering, the transfer agent and registrar for our common stock will be . The transfer agent's address is , and its telephone number is . Our shares of common stock will be issued in uncertificated form only, subject to limited circumstances.

Market Listing

We intend to apply to list our common stock on the NASDAQ Capital Market under the symbol "KRYS."

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has not been a public market for shares of our common stock, and we cannot predict the effect, if any, that market sales of shares of our common stock or the availability of shares of our common stock for sale will have on the market price of our common stock prevailing from time to time. Nevertheless, sales of substantial amounts of our common stock, including shares issued upon exercise of outstanding options, in the public market following this offering could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through the sale of our equity securities.

Upon the completion of this offering, assuming no exercise of the underwriters' option to purchase additional shares, we will have a total of shares of our common stock outstanding, assuming: (i) the automatic conversion of shares of our preferred stock outstanding as of March 31, 2017 into 39,914 shares of our common stock effective immediately prior to the completion of this offering; (ii) the sale and issuance of shares of our common stock in this offering at the initial public offering price of $ per share; (iii) the conversion of all of our outstanding convertible promissory notes and accrued interest into shares of our common stock immediately prior to the completion of this offering; (iv) a a 1-to- forward stock split, which will occur immediately prior to the completion of this offering; and (v) no outstanding options are exercised. Of these outstanding shares, all of the shares of common stock sold in this offering will be freely tradable, except that any shares purchased in this offering by our affiliates, as that term is defined in Rule 144 under the Securities Act, would only be able to be sold in compliance with the Rule 144 limitations described below.

The remaining outstanding shares of our common stock will be deemed "restricted securities" as defined in Rule 144. Restricted securities may be sold in the public market only if they are registered under the Securities Act or if they qualify for an exemption from registration under Rule 144 or Rule 701 promulgated under the Securities Act, which rules are summarized below. In addition, all of our security holders have entered into lock-up agreements with the underwriters under which they have agreed, subject to specific exceptions, not to sell any of our stock for at least 180 days following the date of this prospectus, as described below. As a result of these agreements, subject to the provisions of Rule 144 or Rule 701, shares will be available for sale in the public market as follows:

- beginning on the date of this prospectus, all of the shares sold in this offering will be immediately available for sale in the public market (except as described above); and
- beginning 181 days after the date of this prospectus, additional shares will become eligible for sale in the public market, of which shares will be held by affiliates and subject to the volume and other restrictions of Rule 144 and Rule 701 as described below.

Lock-Up Agreements

All of our directors, executive officers and our security holders are subject to lock-up agreements that, subject to certain exceptions, prohibit them from directly or indirectly offering, pledging, selling, contracting to sell, selling any option or contract to purchase, purchasing any option or contract to purchase, granting any option, right or warrant to purchase or otherwise transferring or disposing of any shares of our common stock, options to acquire shares of our common stock or any securities convertible into or exercisable or exchangeable for common stock, whether now owned or hereafter acquired, or entering into any swap or any other agreement or any transaction that transfer, in whole or in part, directly or indirectly, the economic consequence of ownership, for a period of 180 days following the date of this prospectus, without the prior written consent of Ladenburg Thalmann & Co. Inc. These agreements are described in the section entitled "Underwriting."

Rule 144

In general, under Rule 144 as currently in effect, once we have been subject to public company reporting requirements for at least 90 days, a person who is not deemed to have been one of our affiliates

for purposes of the Securities Act at any time during the 90 days preceding a sale and who has beneficially owned the shares proposed to be sold for at least six months, including the holding period of any prior owner other than our affiliates, is entitled to sell those shares without complying with the manner of sale, volume limitation or notice provisions of Rule 144, subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than our affiliates, then that person would be entitled to sell those shares without complying with any of the requirements of Rule 144.

In general, under Rule 144, as currently in effect, our affiliates or persons selling shares on behalf of our affiliates are entitled to sell upon expiration of the lock-up agreements described above, within any three-month period, a number of shares that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately shares immediately after this offering; or
- the average weekly trading volume of our common stock during the four calendar weeks preceding the filing of a notice on Form 144 with respect to that sale.

Sales under Rule 144 by our affiliates or persons selling shares on behalf of our affiliates are also subject to certain manner of sale provisions and notice requirements and to the availability of current public information about us.

Rule 701

Rule 701 generally allows a stockholder who purchased shares of our common stock pursuant to a written compensatory plan or contract and who is not deemed to have been an affiliate of our company during the immediately preceding 90 days to sell these shares in reliance upon Rule 144, but without being required to comply with the public information, holding period, volume limitation or notice provisions of Rule 144. Rule 701 also permits affiliates of our company to sell their Rule 701 shares under Rule 144 without complying with the holding period requirements of Rule 144. All holders of Rule 701 shares, however, are required by that rule to wait until 90 days after the date of this prospectus before selling those shares pursuant to Rule 701 and are subject to the lock-up agreements described above.

Equity Incentive Options

In connection with this offering, we intend to file a registration statement on Form S-8 under the Securities Act covering all of the shares of our common stock subject to outstanding options and the shares of our common stock reserved for issuance under our stock plans. We expect to file this registration statement as soon as permitted under the Securities Act. However, the shares registered on Form S-8 may be subject to the volume limitations and the manner of sale, notice and public information requirements of Rule 144 and will not be eligible for resale until expiration of the lock-up agreements to which they are subject.

MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS TO NON-U.S. HOLDERS

The following is a summary of the material U.S. federal income tax consequences applicable to non-U.S. holders (as defined below) with respect to the acquisition, ownership and disposition of shares of our common stock, but does not purport to be a complete analysis of all potential tax considerations related thereto. This summary is based on current provisions of the Internal Revenue Code of 1986, as amended, or the Code, final, temporary or proposed Treasury regulations promulgated thereunder, administrative rulings and judicial opinions, all of which are subject to change, possibly with retroactive effect. We have not sought any ruling from the U.S. Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions.

This summary is limited to non-U.S. holders who purchase shares of our common stock issued pursuant to this offering and who hold such shares of our common stock as capital assets (within the meaning of Section 1221 of the Code).

This discussion does not address all aspects of U.S. federal income taxation that may be important to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances, nor does it address the potential application of the Medicare contribution tax, any aspects of U.S. federal estate or gift tax laws, or tax considerations arising under the laws of any non-U.S., state or local jurisdiction. This discussion also does not address tax considerations applicable to a non-U.S. holder subject to special treatment under the U.S. federal income tax laws, including without limitation:

- banks, insurance companies or other financial institutions;
- partnerships or other pass-through entities;
- tax-exempt organizations;
- tax-qualified retirement plans;
- dealers in securities or currencies;
- traders in securities that elect to use a mark-to-market method of accounting for their securities
- holdings;
- U.S. expatriates and certain former citizens or long-term residents of the United States;
- controlled foreign corporations;
- passive foreign investment companies;
- persons that own, or have owned, actually or constructively, more than 5% of our common stock; and
- persons that will hold common stock as a position in a hedging transaction, "straddle" or "conversion transaction" for tax purposes.

If a partnership (or entity classified as a partnership for U.S. federal income tax purposes) is a beneficial owner of shares of our common stock, the tax treatment of a partner in the partnership (or member in such other entity) will generally depend upon the status of the partner and the activities of the partnership. Any partner in a partnership holding shares of our common stock (and such partnership) should consult their own tax advisors.

PROSPECTIVE INVESTORS ARE URGED TO CONSULT THEIR TAX ADVISORS WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERAL INCOME TAX LAWS TO THEIR PARTICULAR SITUATIONS AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF SHARES OF OUR COMMON STOCK ARISING UNDER THE U.S. FEDERAL ESTATE OR GIFT TAX RULES OR UNDER THE LAWS OF ANY STATE, LOCAL, NON-U.S. OR OTHER TAXING JURISDICTION OR UNDER ANY APPLICABLE TAX TREATY.

Definition of Non-U.S. Holder

For purposes of this summary, a "non-U.S. holder" is any beneficial owner of shares of our common stock (other than a partnership or other entity treated as a partnership for U.S. federal income tax purposes) that is not a U.S. person. A "U.S. person" is any of the following:

- an individual citizen or resident of the United States;
- a corporation created or organized in or under the laws of the United States, any state thereof or the District of Columbia(or entity treated as such for U.S. federal income tax purposes);
- an estate, the income of which is includible in gross income for U.S. federal income tax purposes regardless of its source; or
- a trust if: (i) a court within the United States is able to exercise primary supervision over the administration of the trust and one or more U.S. persons have the authority to control all substantial decisions of the trust; or (ii) it has a valid election in effect under applicable Treasury regulations to be treated as a U.S. person.

Distributions on Our Common Stock

As described in the section titled "Dividend Policy," we currently do not anticipate paying dividends on our common stock in the foreseeable future. If, however, we make cash or other property distributions on our common stock (other than certain pro rata distributions of shares of our common stock), such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current earnings and profits for that taxable year or accumulated earnings and profits, as determined under U.S. federal income tax principles. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and will first be applied against and reduce a holder's adjusted tax basis in the shares of our common stock, but not below zero. Any excess will be treated as gain realized on the sale or other disposition of shares of our common stock and will be treated as described under the section titled "—Gain on Sale or Other Disposition of Shares of Our Common Stock" below.

Dividends paid to a non-U.S. holder of our common stock generally will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends, or such lower rate specified by an applicable income tax treaty. To receive the benefit of a reduced treaty rate, a non-U.S. holder must furnish to us or our paying agent a valid IRS Form W-8BEN or W-8BEN-E (or applicable successor form) certifying, under penalties of perjury, such holder's qualification for the reduced rate. This certification must be provided to us or our paying agent prior to the payment of dividends and must be updated periodically.

If a non-U.S. holder holds shares of our common stock in connection with the conduct of a trade or business in the United States, and dividends paid on shares of our common stock are effectively connected with such holder's U.S. trade or business (and, if required by an applicable income tax treaty, are attributable to a permanent establishment maintained by the non-U.S. holder in the United States), the non-U.S. holder will be exempt from the aforementioned U.S. federal withholding tax. To claim the exemption, the non-U.S. holder must furnish to us or our paying agent a properly executed IRS Form W-8ECI (or applicable successor form).

Such effectively connected dividends generally will be subject to U.S. federal income tax on a net income basis at the regular graduated U.S. federal income tax rates in the same manner as if such holder were a resident of the United States. A non-U.S. holder that is a non-U.S. corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of a portion of its effectively connected earnings and profits for the taxable year. Non-U.S. holders should consult any applicable income tax treaties that may provide for different rules.

A non-U.S. holder that claims exemption from withholding or the benefit of an applicable income tax treaty generally will be required to satisfy applicable certification and other requirements prior to the

distribution date. Non-U.S. holders that do not timely provide us or our paying agent with the required certification, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. Non-U.S. holders should consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty or applicability of other exemptions from withholding.

Gain on Sale or Other Disposition of Shares of Our Common Stock

Subject to the discussion below regarding backup withholding, a non-U.S. holder generally will not be subject to U.S. federal income tax on any gain realized upon the sale or other disposition of shares of our common stock unless:

- the gain is effectively connected with a trade or business carried on by the non-U.S. holder in the United States and, if required by an applicable income tax treaty, the gain is attributable to a permanent establishment of the non-U.S. holder maintained in the United States;
- the non-U.S. holder is an individual present in the United States for 183 days or more in the taxable year of disposition and certain other requirements are met; or
- we are or have been a U.S. real property holding corporation, or a USRPHC, for U.S. federal income tax purposes at any time within the shorter of the five-year period preceding the disposition and the non-U.S. holder's holding period for the shares of our common stock, and our common stock has ceased to be traded on an established securities market prior to the beginning of the calendar year in which the sale or other disposition occurs. The determination of whether we are a USRPHC depends on the fair market value of our U.S. real property interests relative to the fair market value of our other trade or business assets and our foreign real property interests.

We believe we currently are not, and we do not anticipate becoming, a USRPHC for U.S. federal income tax purposes.

Gain described in the first bullet point above will be subject to U.S. federal income tax on a net income basis at regular graduated U.S. federal income tax rates generally in the same manner as if such holder were a resident of the United States. A non-U.S. holder that is a non-U.S. corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of a portion of its effectively connected earnings and profits for the taxable year. Non-U.S. holders should consult any applicable income tax treaties that may provide for different rules.

Gain described in the second bullet point above will be subject to U.S. federal income tax at a flat 30% rate (or such lower rate specified by an applicable income tax treaty) but may be offset by U.S. source capital losses (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses. Non-U.S. holders should consult any applicable income tax treaties that may provide for different rules.

Backup Withholding and Information Reporting

Generally, we must report annually to the IRS and to each non-U.S. holder the amount of dividends paid to, and the tax withheld with respect to, each non-U.S. holder. This information also may be made available under a specific treaty or agreement with the tax authorities in the country in which the non-U.S. holder resides or is established. Backup withholding, currently at a 28% rate, generally will not apply to distributions to a non-U.S. holder of shares of our common stock provided the non-U.S. holder furnishes to us or our paying agent the required certification as to its non-U.S. status, such as by providing a valid IRS Form W-8BEN, IRS Form W-8BEN-E, or IRS Form W-8ECI, or certain other requirements are met. Notwithstanding the foregoing, backup withholding may apply if either we or our paying agent has actual knowledge, or reason to know, that the holder is a U.S. person that is not an exempt recipient.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules may be allowed as a refund or a credit against a non-U.S. holder's U.S. federal income tax liability, provided the required information is timely furnished to the IRS.

Foreign Account Tax Compliance Act

Legislation and administrative guidance, commonly referred to as "FATCA", may impose a 30% withholding tax on any dividends paid after July 1, 2014 and the proceeds of a sale of our common stock paid after December 31, 2018 to a "foreign financial institution", as specially defined under such rules, and certain other foreign entities, unless various information reporting and due diligence requirements (generally relating to ownership by U.S. persons of interests in, or accounts with, those entities) have been met or an exemption applies. If FATCA withholding is imposed, a beneficial owner that is not a foreign financial institution generally will be entitled to a refund of any amounts withheld by filing a U.S. federal income tax return (which may entail significant administrative burden). Prospective investors should consult their tax advisors regarding FATCA.

UNDERWRITING

We have entered into an underwriting agreement dated , 2017, with Ladenburg Thalmann & Co. Inc., as the underwriter and the sole book-running manager of this offering. Subject to the terms and conditions of the underwriting agreement, the underwriter has agreed to purchase the number of our securities set forth opposite its name below.

Underwriter	Number of shares
Ladenburg Thalmann & Co. Inc.	

We have been advised by the underwriter that it proposes to offer the shares directly to the public at the public offering price set forth on the cover page of this prospectus. Any shares sold by the underwriter to securities dealers will be sold at the public offering price less a selling concession not in excess of per share. The underwriter may allow, and these selected dealers may re-allow, a concession of not more than per share to other brokers and dealers.

The underwriting agreement provides that the underwriter's obligation to purchase the shares we are offering is subject to the terms and conditions described therein.

No action has been taken by us or the underwriter that would permit a public offering of the shares in any jurisdiction where action for that purpose is required. None of our shares included in this offering may be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sales of any of the shares offered hereby be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons who receive this prospectus are advised to inform themselves about and to observe any restrictions relating to this offering of shares and the distribution of this prospectus. This prospectus is neither an offer to sell nor a solicitation of any offer to buy the shares in any jurisdiction where that would not be permitted or legal.

The underwriter has advised us that they do not intend to confirm sales to any accounts over which they exercise discretionary authority.

Over-Allotment Option

We have granted to the underwriter an option, exercisable for 30 days from the date of this prospectus, to purchase up to additional shares from us at the public offering price set forth on the cover page of this prospectus, less the underwriting discount and commission. The underwriter may exercise this option solely for the purpose of covering over-allotments, if any, made in connection with the offering of the shares offered by this prospectus.

Underwriting Discount and Expenses

The following table shows the public offering price, underwriting discount and commission, and proceeds before expenses to us. The information assumes either no exercise or full exercise of the option we granted to the underwriter to purchase additional shares.

		Total	
	Per share	No exercise	Full exercise
Public offering price	$	$	$
Underwriting discounts and commissions			
Proceeds, before expenses, to us			

We estimate expenses payable by us in connection with this offering, other than the underwriting discounts and commissions referred to above, will be approximately $. We have agreed to reimburse the underwriter for expenses relating to this offering of up to $.

Determination of Offering Price

Prior to the completion of this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the underwriter. Among the factors considered in determining the initial public offering price will be the history and prospects of other companies in the industry in which we compete; our financial information; an assessment of our management and their experience; an assessment of our business potential and earning prospects; the prevailing securities markets at the time of this offering; the recent market prices of, and the demand for, publicly traded shares of generally comparable companies; and other factors deemed relevant. Neither we nor the underwriter can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

Lock-up Agreements

We, all of our officers and directors, and holders of all of our outstanding shares of common stock have agreed, that for a period of 180 days after the date of this prospectus, or the lock-up period, subject to certain limited exceptions described below, we and they will not directly or indirectly, without the prior written consent of the underwriter offer for sale, contract to sell, sell, distribute, grant any option, right or warrant to purchase, pledge, hypothecate or otherwise dispose of, directly or indirectly, any shares of our common stock or any securities convertible into, or exercisable or exchangeable for, shares of our common stock. Certain limited transfers are permitted during the lock-up period if the transferee agrees to these lock-up restrictions. We have also agreed, in the underwriting agreement, to similar lock-up restrictions on the issuance and sale of our securities for 180 days following the closing of this offering, although we will be permitted to issue stock options or stock awards to directors, officers and employees under our existing equity incentive plans. The underwriter may, in its sole discretion and without notice, waive the terms of any of these lock-up agreements.

Stabilization

In connection with this offering, the underwriter may engage in stabilizing transactions and syndicate covering transactions and purchases to cover positions created by short sales.

- Stabilizing transactions permit bids to purchase shares of common stock so long as the stabilizing bids do not exceed a specified maximum, and are engaged in for the purpose of preventing or retarding a decline in the market price of the common stock while the offering is in progress.
- Syndicate covering transactions involve purchases of common stock in the open market after the distribution has been completed in order to cover syndicate short positions. Since there is no over-allotment option, if the underwriter would have a naked short position, it can be closed out only by buying shares in the open market. A naked short position is more likely to be created if the underwriter is concerned that after pricing there could be downward pressure on the price of the shares in the open market that could adversely affect investors who purchase in the offering.

Penalty bids permit the underwriter to reclaim a selling concession from a syndicate member when the security originally sold by the syndicate member is purchased in a stabilizing or syndicate covering transaction to cover syndicate short positions. These stabilizing transactions, syndicate covering transactions and penalty bids may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock in the open market may be higher than it would otherwise be in the absence of these transactions. Neither we nor the underwriter makes any representation or prediction as to the effect that the transactions described above may have on the price of our common stock. These transactions may be effected on the NASDAQ Capital Market, in the over-the-counter market or otherwise and, if commenced, may be discontinued at any time.

Indemnification

We have agreed to indemnify the underwriter and selected dealers against certain liabilities, including certain liabilities arising under the Securities Act, or to contribute to payments that the underwriter or selected dealers may be required to make for these liabilities.

Listing on the NASDAQ Capital Market

We intend to apply to list our common stock on the NASDAQ Capital Market under the symbol “KRYS.”

Electronic Distribution

A prospectus in electronic format may be made available on websites maintained by the underwriter, or selling group members, if any, participating in this offering. The underwriter may agree to allocate a number of shares of our common stock for sale to its online brokerage account holders.

Other Relationships

The underwriter and its affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing, and brokerage activities. The underwriter and its affiliates may in the future perform various financial advisory, investment banking, and other services for us, for which they may receive customary fees and commissions. In addition, in the ordinary course of their various business activities, the underwriter and its affiliates may effect transactions for their own account or the accounts of customers, and hold on behalf of themselves or their customers long or short positions in our debt or equity securities or loans, and may do so in the future. The underwriter and its affiliates may also make investment recommendations or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to their customers that they acquire, long or short positions in such securities and instruments.

Selling Restrictions

European Economic Area

In relation to each Member State of the European Economic Area that has implemented the Prospectus Directive (each a Relevant Member State), an offer to the public of any shares of our common stock may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any shares of our common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

- to any legal entity which is a qualified investor as defined in the Prospectus Directive;
- to fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or
- in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares of our common stock shall result in a requirement for the publication by us or the underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive, or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

For the purposes of this provision: (i) the expression an “offer to the public” in relation to any shares of our common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase any shares of our common stock, as the same may

be varied in that Member State by any measure implementing the Prospectus Directive in that Member State; (ii) the expression “Prospectus Directive” means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State; and (iii) the expression “2010 PD Amending Directive” means Directive 2010/73/EU.

United Kingdom

The underwriter has represented and agreed that:

- it has only communicated or caused to be communicated, and will only communicate or cause to be communicated, an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act 2000 (FSMA)) received by it in connection with the issue or sale of the shares of our common stock in circumstances in which Section 21(1) of the FSMA does not apply to us; and
- it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares of our common stock in, from, or otherwise involving the United Kingdom.

LEGAL MATTERS

The validity of the shares of common stock offered hereby will be passed upon for us by Morrison & Foerster LLP, San Francisco, California. The underwriters are being represented by Goodwin Procter LLP, New York, New York, in connection with this offering.

EXPERTS

The financial statements of Krystal Biotech, Inc. as of and for the year ended December 31, 2016 included in this prospectus and in the registration statement have been audited by Mayer Hoffman McCann P.C., independent registered public accounting firm, and are included in reliance on their report given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock offered by this prospectus. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement, some of which is contained in exhibits to the registration statement as permitted by the rules and regulations of the SEC. For further information with respect to us and our common stock, we refer you to the registration statement, including the exhibits filed as a part of the registration statement. Statements contained in this prospectus concerning the contents of any contract or any other document is not necessarily complete. If a contract or document has been filed as an exhibit to the registration statement, please see the copy of the contract or document that has been filed. Each statement in this prospectus relating to a contract or document filed as an exhibit is qualified in all respects by the filed exhibit. You may obtain copies of this information by mail from the Public Reference Section of the SEC, 100 F Street, N.E., Room 1580, Washington, D.C. 20549, at prescribed rates. You may obtain information on the operation of the public reference rooms by calling the SEC at 1-800-SEC-0330. The SEC also maintains an Internet website that contains reports, proxy statements and other information about issuers, like us, that file electronically with the SEC. The address of that website is www.sec.gov.

As a result of this offering, we will become subject to the information and reporting requirements of the Securities Exchange Act of 1934 and, in accordance with this law, will file periodic reports, proxy statements and other information with the SEC. These periodic reports, proxy statements and other information will be available for inspection and copying at the SEC's public reference facilities and the website of the SEC referred to above. We also maintain a website at www.krystalbio.com. Upon completion of this offering, you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Information contained on our website is not a part of this prospectus and the inclusion of our website address in this prospectus is an inactive textual reference only.

Krystal Biotech, Inc. (formerly Krystal Biotech, LLC)
Index to Financial Statements

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders
Krystal Biotech, Inc.

We have audited the accompanying balance sheet of **Krystal Biotech, Inc.** (the "Company") as of December 31, 2016, and the related statements of operations, convertible preferred stock and stockholders' and members' equity (deficit), and cash flows for the year ended December 31, 2016. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audit provides a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of **Krystal Biotech, Inc.** as of December 31, 2016, and the results of its operations and its cash flows for the year ended December 31, 2016, in conformity with U.S. generally accepted accounting principles.

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has experienced losses since inception and is dependent on future financing to fund its planned operations. These conditions raise substantial doubt about its ability to continue as a going concern. Management's plans regarding these matters are also described in Note 1. The accompanying financial statements do not include any adjustments that might result should the Company be unable to continue as a going concern.

/s/ Mayer Hoffman McCann P.C.

San Diego, California
July 14, 2017

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

(In thousands, except per share and per unit data)	December 31, 2016	March 31, 2017	Pro Forma March 31, 2017
		(unaudited)	
Assets			
Current assets			
Cash	$ 1,923	$ 1,959	$ 1,959
Prepaid research and development expenses	246	173	173
Total current assets	2,169	2,132	2,132
Property and equipment, net	13	19	19
Total assets	$ 2,182	$ 2,151	$ 2,151
Liabilities, Convertible Preferred Stock and Stockholders' and Members' Equity (Deficit)			
Current liabilities			
Accounts payable	$ 42	$ 58	$ 58
Accrued expenses	1	27	27
Total current liabilities	43	85	85
Accrued interest	7	37	—
Related party convertible promissory notes	698	698	—
Convertible promissory notes	1,145	1,445	—
Total liabilities	1,893	2,265	85
Commitments and contingencies (Note 7)			
Convertible preferred stock			
Convertible preferred stock; $0.00001 par value; no shares authorized, issued, and outstanding at December 31, 2016, and 100,000 shares authorized, 39,914 issued and outstanding at March 31, 2017 (unaudited) (aggregate liquidation preference of $1,406), and no shares issued and outstanding pro forma March 31, 2017 (unaudited)	—	1,406	—
Total convertible preferred stock	—	1,406	—
Stockholders' and members' equity (deficit)			
Common stock; $0.00001 par value; no shares authorized, issued or outstanding at December 31, 2016, and 10,000,000 shares authorized, 775,752 shares issued and outstanding at March 31, 2017 (unaudited), and 10,000,000 shares authorized, 932,512 shares issued and outstanding pro forma March 31, 2017 (unaudited)	—	—	—
Common units; no par value; 775,752 units authorized, issued, and outstanding at December 31, 2016, no common units authorized, issued, and outstanding at March 31, 2017 (unaudited), or pro forma March 31, 2017 (unaudited)	—	—	—
Preferred units; no par value; 39,914 units authorized, issued, and outstanding at December 31, 2016 (aggregate liquidation preference of $1,406), no preferred units authorized, issued, and outstanding at March 31, 2017 (unaudited), or pro forma March 31, 2017 (unaudited)	1,406	—	—
Additional paid-in capital	33	124	6,156
Accumulated deficit	(1,150)	(1,644)	(4,090)
Total stockholders' and members' equity (deficit)	289	(1,520)	2,066
Total liabilities, convertible preferred stock and stockholders' and members' equity (deficit)	$ 2,182	$ 2,151	$ 2,151

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

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

(In thousands, except shares, units, and per share and per unit data)	Year Ended December 31, 2016	Three Months Ended March 31, 2016 (unaudited)	2017 (unaudited)	2017 (unaudited, pro forma)
Revenues				
Revenues	$ —	$ —	$ —	$ —
Total revenues	—	—	—	—
Expenses				
Research and development	741	—	319	319
General and administrative	402	—	146	146
Total operating expenses	1,143	—	465	465
Loss from operations	(1,143)	—	(465)	(465)
Other Expense				
Interest expense, net	(7)	—	(29)	(2,475)
Total other expense	(7)	—	(29)	(2,475)
Net loss	(1,150)	—	(494)	(2,940)
Net loss applicable to stockholders and members	$ (1,150)	$ —	$ (494)	$ (2,940)
Basic and diluted net loss per common share and common unit	$ (5.89)	$ —	$ (0.64)	$ (3.79)
Weighted-average basic and diluted common shares and common units (Note 2)	194,998	—	775,752	775,752
Pro forma basic and diluted net loss per common share and common unit (unaudited) (Note 2)			$ (0.50)	$ (3.13)
Pro forma weighted-average basic and diluted common shares and common units (unaudited) (Note 2)			930,826	930,826

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

Krystal Biotech, Inc. (formerly Krystal Biotech, LLC)
Statements of Convertible Preferred Stock and Stockholders' and Members' Equity (Deficit)
(In thousands, except units and shares)

	Convertible Preferred Stock		Stockholders' and Members' Equity (Deficit)								Total Stockholders' and Members' Equity (Deficit)
			Common Stock		Common Units		Preferred Units		Additional Paid-in Capital	Accumulated Deficit	
	Shares	Amount	Shares	Amount	Units	Amount	Units	Amount			
Balances at January 1, 2016	—	$ —	—	$ —	—	$ —	—	$ —	$ —	$ —	$ —
Issuance of common units	—	—	—	—	100	100	—	—	—	—	100
Conversion from common units to preferred units	—	—	—	—	(100)	(100)	2,838	100	—	—	—
Issuance of preferred and common units	—	—	—	—	775,752	—	37,076	1,306	—	—	1,306
Stock-based compensation expense	—	—	—	—	—	—	—	—	33	—	33
Net loss	—	—	—	—	—	—	—	—	—	(1,150)	(1,150)
Balances at December 31, 2016	—	$ —	—	$ —	775,752	$ —	39,914	$ 1,406	$ 33	$ (1,150)	$ 289
Conversion of preferred units to preferred stock (unaudited)	39,914	1,406	—	—	—	—	(39,914)	(1,406)	—	—	(1,406)
Conversion of common units to common stock (unaudited)	—	—	775,752	—	(775,752)	—	—	—	—	—	—
Stock-based compensation expense (unaudited)	—	—	—	—	—	—	—	—	91	—	91
Net loss (unaudited)	—	—	—	—	—	—	—	—		(494)	(494)
Balances at March 31, 2017 (unaudited)	39,914	$ 1,406	775,752	$ —	—	$ —	—	$ —	$ 124	$ (1,644)	$ (1,520)
Conversion of convertible preferred stock to common stock (unaudited)	(39,914)	(1,406)	39,914	—	—	—	—	—	1,406	—	1,406
Conversion of convertible promissory notes to common stock (unaudited)	—	—	116,846	—	—	—	—	—	4,626	(2,446)	2,180
Pro Forma Balances at March 31, 2017 (unaudited)	—	$ —	932,512	$ —	—	$ —	—	$ —	$ 6,156	$ (4,090)	$ 2,066

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

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

(In thousands)	Year Ended December 31, 2016	Three Months Ended March 31, 2016	Three Months Ended March 31, 2017
		(unaudited)	
Operating Activities			
Net loss	$ (1,150)	$ —	$ (494)
Adjustments to reconcile net loss to net cash used in operating activities			
Depreciation	2	—	1
Stock-based compensation expense	33	—	91
Non-cash interest expense	7	—	30
(Increase) decrease in			
Prepaid expenses	(246)	—	73
Increase (decrease) in			
Accounts payable	42	—	16
Accrued expenses	1	—	26
Net cash used in operating activities	(1,311)	—	(257)
Investing Activities			
Purchases of property and equipment	(15)	—	(7)
Net cash used in investing activities	(15)	—	(7)
Financing Activities			
Proceeds from the issuance of convertible promissory notes	1,145	—	300
Proceeds from the issuance of related party convertible promissory notes	698	—	—
Issuance of common stock and common units	100	100	—
Issuance of preferred stock and preferred units	1,306	—	—
Net cash provided by financing activities	3,249	100	300
Net increase in cash	1,923	100	36
Cash at beginning of period	—	—	1,923
Cash at end of period	$ 1,923	$ 100	$1,959
Supplemental Disclosures of Non-Cash Operating, Investing and Financing Activities			
Conversion of common units to preferred units	$ 100	$ —	$ —
Conversion of preferred units to preferred stock	$ —	$ —	$1,306
Cash paid for interest	$ —	$ —	$ —
Cash paid for taxes	$ —	$ —	$ —

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

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

1. Nature of Business

Krystal Biotech, Inc. (the "Company") was formed on December 20, 2015 in the State of California as Krystal Biotech, LLC, but 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. The Company seeks to use gene therapy to develop novel treatments for patients suffering from dermatological diseases. The Company is currently conducting preclinical studies evaluating its lead product candidate, KB103, which is intended to treat dystrophic epidermolysis bullosa, or DEB, a rare and severe genetic disease for which there is currently no approved treatment.

Liquidity and Risks

As of December 31, 2016, the Company generated an accumulated deficit of $1.2 million since inception and will require substantial additional capital to fund its research and development. As of March 31, 2017 (unaudited), the accumulated deficit was $1.6 million. The Company believes that its cash of approximately $2.0 million as of March 31, 2017 (unaudited) will be sufficient to allow the Company to fund its operations into at least the first quarter of 2018. 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 seeking to complete an initial public offering ("IPO") of its common stock. Upon the closing of a qualified public offering, the Company's outstanding convertible promissory notes and convertible preferred stock will automatically convert into shares of common stock (Note 8).

In the event the Company does not complete an IPO, the Company expects to seek additional funding through private financings, debt financing, collaboration agreements, or government grants. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into collaboration arrangements or obtain government grants. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to obtain funding, the Company could be forced to delay, reduce, or eliminate its research and development programs, product candidate expansion, or commercialization efforts, which could adversely affect its business prospects. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

The foregoing matters give rise to substantial doubt about the Company's ability to continue as a going concern. The accompanying financial statements do not include any adjustments that might result from the outcome of this uncertainty.

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 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 Interim Financial Information

The accompanying balance sheet as of March 31, 2017, the statements of operations and statements of cash flows for the three months ended March 31, 2016 and 2017, the statement of convertible preferred stock and stockholders' and members equity (deficit) for the three months ended March 31, 2017 and the related information contained within the notes to the financial statements are unaudited. The interim 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 March 31, 2017 and results of its operations and its cash flows for the three months ended March 31, 2016 and 2017. The results for the three months ended March 31, 2017 are not necessarily indicative of results to be expected for the year ending December 31, 2017 or any other interim or future period.

Unaudited Pro Forma Information

On July 13, 2017, the Company's board of directors authorized the Company to file a registration statement with the Securities and Exchange Commission ("SEC") permitting the Company to sell shares of its common stock to the public. Upon the closing of a qualified (as defined in the Company's Articles of Incorporation) initial public offering ("IPO") or otherwise upon the election of the holders of the specified percentage of Preferred Stock, all of the Company's outstanding convertible preferred stock will automatically convert into common stock and will meet the GAAP criteria for equity classification. The unaudited pro forma balance sheet and statement of convertible preferred stock and stockholders' and member's equity (deficit) as of March 31, 2017 reflect the assumed conversion of all of the outstanding shares of convertible preferred stock ("Preferred Stock") and the convertible promissory notes (the "Notes") into shares of common stock upon the completion of this proposed offering.

Unaudited pro forma net loss per share attributable to common stockholders is computed using the weighted-average number of common shares outstanding after giving effect to the conversion of all the outstanding Preferred Stock and the convertible promissory notes, plus accrued interest, into shares of common stock as if such conversion had occurred at the beginning of the period presented, or the date of original issuance, if later. As the year ended December 31, 2016 and the three months ended March 31, 2017 resulted in net losses, there is no income allocation required under the two-class method or dilution attributed to pro forma weighted average shares outstanding in the calculation of pro forma diluted loss per share attributable to common stockholders. The three months ended March 31, 2016, did not result in net operating losses because the Company began operations in the second quarter of 2016.

As noted above, the unaudited pro forma information reflects the automatic conversion, at the closing of an IPO of the Company's common stock, of all outstanding shares of Preferred Stock and the Notes into shares of common stock. The conversion has been reflected assuming a 1-to-1 conversion ratio for the Preferred Stock and the conversion of the Notes has been reflected assuming conversion at a price of $18.64 per share. See Note 8 for further discussion of the Preferred Stock conversion feature, as well as a discussion of the rights and preferences of the Preferred Stock and see Note 5 for further discussion of the conversion features of the Notes.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could materially differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the financial statements. Estimates are used in the following areas, 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

The financial instrument that potentially subjects the Company to concentrations of credit risk is cash held in a depository account. The Company's cash is held with a financial institution that management believes is creditworthy. These amounts at times may exceed federally 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.

Deferred Issuance Costs

Deferred public offering costs, which primarily consist of direct, incremental legal and accounting fees relating to the IPO, are capitalized within other assets. The deferred issuance costs will be offset against IPO proceeds upon the consummation of the offering. In the event the offering is terminated, deferred offering costs will be expensed. The Company has incurred no IPO costs as of March 31, 2017 (unaudited).

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 year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited). The Company evaluates transfers between levels at the end of each reporting period. There were no transfers of financial instruments between levels during the year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited).

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 financial statements are reasonable estimates of fair value due to their short maturities.

Property and Equipment

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 March 31, 2017 (unaudited).

Convertible Preferred Stock

In accordance with the guidance in FASB ASC Topic 480, *Distinguishing Liabilities from Equity*, outstanding shares of Preferred Stock (Note 8), were classified outside of permanent equity and within temporary equity, as of March 31, 2017 (unaudited) due to their associated redemption features and liquidation preferences.

The Company evaluated its Preferred Stock and determined that they are considered equity hosts 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 financial statements is based on awards that are ultimately expected to vest.

Income Taxes

For the year ended December 31, 2016, the Company was organized in the State of California as a limited liability corporation and was taxed as a partnership for United States income tax purposes and therefore files federal and state flow through income tax returns. As a result, no U.S. current or deferred income tax assets or liabilities are reflected in December 31, 2016 financial statements. The Company's members are obligated to report that member's proportionate share of the Company's taxable income or loss.

For the three months ended March 31, 2017 income taxes are recorded in accordance with FASB ASC Topic 740, *Income Taxes* ("ACS 740"), which provides for deferred taxes using an asset and liability approach. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial reporting and the tax reporting basis of assets and liabilities and are measured using the enacted tax rates and laws that are expected to be in effect when the differences are expected to reverse. The Company provides a valuation allowance against net deferred tax assets unless, based upon the available evidence, it is more likely than not that the deferred tax assets will be realized. The Company has evaluated available evidence and concluded that the Company may not realize the benefit of its deferred tax assets; therefore a valuation allowance has been established for the full amount of the deferred tax assets.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of March 31, 2017 (unaudited), the Company does not have any significant uncertain tax positions.

The Company may recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2016 and March 31, 2017 (unaudited), the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's statements of operations.

Net Loss Per Share Attributable to Common Stockholders

On March 31, 2017, the Company converted from an LLC to a C-corporation. Upon the conversion, each common unit and each preferred unit (Note 8) held were converted into one share of common stock and Preferred Stock, respectively. Common units of the LLC 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 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 were the same for all periods presented.

(In thousands, except share and per share data)	December 31, 2016	March 31, 2017 (unaudited)
Numerator:		
Net loss applicable to common stockholders and members	$ (1,150)	$ (494)
Denominator:		
Weighted-average basic and diluted common shares	194,998	775,752
Basic and diluted net loss per common shares	$ (5.89)	$ (0.64)
Pro forma basic and diluted net loss per common share and common unit		$ (0.50)

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 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 year ended December 31, 2016, the three months ended March 31, 2017 (unaudited) and the three months ended March 31, 2017 (unaudited) (pro forma), comprehensive loss was equal to the net loss.

Recent Accounting Pronouncements

In August 2014, the FASB issued *ASU 2014-15 Disclosures of Uncertainties about an Entity’s Ability to Continue as a Going Concern* (“ASU 2014-15”). The standard requires an evaluation of whether there are

conditions or events, considered in the aggregate, that raise substantial doubt about an entity's ability to continue as a going concern. Substantial doubt about an entity's ability to continue as a going concern exists when relevant conditions and events, considered in the aggregate, indicate that it is probable that the entity will be unable to meet its obligations as they become due within one year after the date that the financial statements are issued. ASU 2014-15 is effective for annual periods ending after December 15, 2016 and for interim periods within annual periods beginning after December 15, 2016. The Company has adopted this standard as of December 31, 2016 and the adoption did not have a material impact, refer to Note 1 for future details regarding the Company's liquidity.

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 interim 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 financial statements and related disclosures.

Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the date the financial statements are available to be issued for potential recognition or disclosure in the financial statements.

3. Cash

As of December 31, 2016 and March 31, 2017 (unaudited), cash balances consist of a single depository account. Cash balances at December 31, 2016 and March 31, 2017 (unaudited) were $1.9 million and $2.0 million, respectively.

4. Property and Equipment, Net

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

	December 31, 2016	March 31, 2017 (unaudited)
Computer equipment and software	$ 8	$ 9
Laboratory equipment	7	13
Total property and equipment	15	22
Accumulated depreciation	(2)	(3)
Property and equipment, net	$ 13	$ 19

Depreciation expense was $2 thousand, $0 and $1 thousand for the year ended December 31, 2016 and the three months ended March 31, 2016 and 2017 (unaudited), respectively.

5. Convertible Promissory Notes

General

On November 16, 2016, the Company executed a Note Purchase Agreement (the "Agreement") for the issuance of convertible promissory notes (the "Notes"). The Notes bear interest at a rate of 6% per annum, which is accrued based on a 365 day year and mature, unless sooner paid or converted, principal plus unpaid accrued interest 18 months following the date of the Agreement or May 14, 2018. The Notes become immediately due and payable in the event of an occurrence of default by the Company.

Conversion Features

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.

In the event that the Company shall issue and sell preferred units in the Company, or if the Company has converted into corporate form, shares of the preferred stock of the Company (in either case, the "Financing Securities"), to investors for aggregate proceeds to the Company of not less than $5.0 million, including amounts outstanding under the Notes, then the Notes shall be automatically converted, at the initial closing of the financing event, into a number of units of Financing Securities equal to the quotient 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 Financing Securities; or (ii) the target valuation of $16.0 million divided by the fully diluted units or shares immediately prior to closing of the financing event.

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

As of December 31, 2016 and March 31, 2017 (unaudited), the Company had outstanding balances related to the Notes of $1.8 million and $2.1 million, respectively. Additionally, as of December 31, 2016 and March 31, 2017 (unaudited), the Company had accrued interest related to the Notes of $7 thousand and $37 thousand, respectively.

Interest expense related to the Notes was $7 thousand, $0 and $30 thousand for the year ended December 31, 2016 and the three months ended March 31, 2016 and 2017 (unaudited), respectively.

6. Related Party Convertible Promissory Notes

On November 14, 2016, the Company issued a convertible promissory note, in the amount of $250 thousand, under the Agreement (Note 5) to a party related to a director of the Company. The terms of the Notes issued to the related party were at an arms-length and identical to the Notes issued and fully disclosed in Note 5.

On December 27, 2016, the Company issued a convertible promissory note, in the amount of $448 thousand, under the Agreement (Note 5) to a party related to two executive officers and directors of the Company. The terms of the Notes issued to the related party were at an arms-length and identical to the Notes issued and fully disclosed in Note 5.

As of December 31, 2016 and March 31, 2017 (unaudited), accrued interest on related party convertible promissory notes was $2 thousand and $12 thousand, respectively.

Interest expense related to the related party convertible promissory notes was $2 thousand, $0 and $10 thousand for the year ended December 31, 2016, and the three months ended March 31, 2016 and 2017 (unaudited), respectively.

7. Commitments and Contingencies

Significant Contracts and Agreements

Lease Agreements

In May 2016, the Company signed an operating lease for laboratory and office space that commenced in June 2016 and expires on October 31, 2017 (the "2016 Lease"). In June 2016, the Company entered into a lease amendment (the "First Amendment to the 2016 Lease") for office and laboratory space currently occupied under the 2016 Lease. The First Amendment to the 2016 Lease did not change the Company's overall obligation under the lease, but did change the timing of the payment of the obligation from one

single payment to 17 equal monthly installments. On February 27, 2017, the Company entered into the second amendment to the lease (the "Second Amendment to the 2016 Lease"), which extended the expiration date of the lease to October 31, 2018.

As of March 31, 2017 (unaudited), future minimum operating lease payments and future minimum payments to be received from under non-cancelable subleases were as follows (in thousands):

	Operating Leases
2017 (remainder of year)	$ 75
2018	91
2019	—
2020	—
2021	—
Thereafter	—
Future minimum operating lease payments	166
Less: minimum payments to be received from non-cancelable subleases	(34)
Total minimum lease payments, net	$ 132

The Company recorded $54 thousand, $0 and $19 thousand in rent expense for the year ended December 31, 2016 and the three months ended March 31, 2016 and 2017 (unaudited), respectively.

8. Capitalization

Conversion to C-Corporation

On March 31, 2017 (unaudited), the Company converted from an LLC to a C-Corporation. Upon the conversion, the preferred units and the common units were converted on a one-for-one basis into preferred stock and common stock, respectively. After the conversion, the Company had authorized 100,000 shares of Preferred Stock, $0.00001 par value per share, of which 39,914 shares were issued and outstanding and 10,000,000 authorized shares of common stock, par value $0.00001 per share, of which 775,752 shares were issued and outstanding.

General

As of March 31, 2017 (unaudited), the authorized capital stock of the Company included 10,000,000 shares of common stock, par value $0.00001 per share, 775,752 shares issued and outstanding and 100,000 shares of Preferred Stock par value $0.00001 per share, of which, 39,914 shares were issued and outstanding.

As of December 31, 2016, the Company was an LLC and had issued 39,914 preferred units and 775,752 common units.

Preferred Units and Preferred Stock

On April 15, 2016, the Company authorized and issued 100 Member Units for aggregate proceeds of $100 thousand. On September 30, 2016, the Company converted all of the Member Units into 2,838 Preferred Units at an issue price of $35.26 (the "Original Issue Price") per share plus 775,752 Common Units, and issued an additional 21,410 Preferred Units at the Original Issue Price for aggregate proceeds of $754 thousand. On December 27, 2016, the Company issued an additional 15,666 Preferred Units at the Original Issue Price for aggregate proceeds of $552 thousand. On March 31, 2017 (unaudited), all of the Preferred Units were converted into Preferred Stock on a one-for-one basis. As of December 31, 2016 and March 31, 2017 (unaudited), the Company had balances of Preferred Units and Preferred Stock of $1.4 million 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 1:1, 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 of Directors 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 of Directors, 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 March 31, 2017 (unaudited), 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 775,752 Common Units. On March 31, 2017 (unaudited), in connection with the conversion of the LLC to a C-Corporation, all of the Common Units were converted, on a one-for-one basis, into 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 of Directors 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 of Directors, 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 March 31, 2017 (unaudited), 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.

9. 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 material and supplies and 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 drug product is approximately $2.0 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 $11 thousand, $0 and $31 thousand for the year ended December 31, 2016 and the three months ended March 31, 2016 and 2017 (unaudited) respectively.

10. 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 42,105 incentive units to purchase common units.

The 2016 Plan provides 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 (unaudited), the Board of Directors adopted the 2017 Stock Incentive Plan (the "2017 Plan") which authorized the issuance of up to 42,900 shares of the Company's common stock under the plan. Commensurate with the opening of the 2017 Plan, all 25,263 outstanding incentive units granted under the 2016 Plan were converted into 25,263 options to purchase the Company's common stock under the 2017 Plan. As of March 31, 2017, the 2016 Plan was closed and there were no incentive units outstanding.

The 2017 Plan provides for the issuance of stock options, restricted stock awards and unrestricted stock awards to employees, members of the Board of Directors, 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 of Directors. 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 expires in March 31, 2027, 10 years from the date it was approved by the Board of Directors.

The Company granted 31,579 stock options through December 31, 2016, to consultants and board members, which are included in the following table. The options generally vest 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 stock option activity under the 2016 Plan:

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value(1)
Outstanding at January 1, 2016	—			
Granted	31,579	$ 11.07		$ —
Exercised	—			
Cancelled or forfeited	—			
Outstanding at December 31, 2016	31,579	$ 11.07	9.7	$ 338
Granted (unaudited)	—	—		
Exercised (unaudited)	—	—		
Cancelled or forfeited (unaudited)	(6,316)	11.07		
Outstanding at March 31, 2017 (unaudited)	25,263	$ 11.07	9.5	$ 540
Exercisable at December 31, 2016	—	$ —	—	$ —
Vested at December 31, 2016	—	$ —	—	$ —
Exercisable at March 31, 2017 (unaudited)	2,105	$ 11.07	9.6	$ 45
Vested at March 31, 2017 (unaudited)	2,105	$ 11.07	9.6	$ 45

(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 December 31, 2016 and March 31, 2017 (unaudited).

As of December 31, 2016 and March 31, 2017 (unaudited), 10,526 and 17,637 shares of common stock, respectively, were available for future grants under the Plan. The weighted-average grant date fair value of options granted to employees and non-employees during the year ended December 31, 2016 was $7.77. There were no options granted during the three months ended March 31, 2017 (unaudited). During the year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited), there were no stock options exercised.

Stock-based compensation expense for the year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited) relates solely to stock options granted under the Plan. The Company has recorded aggregate stock-based compensation expense related to the issuance of stock option awards to non-employees in the statement of operations for the year ended December 31, 2016 and three months ended March 31, 2017 (unaudited) as follows (in thousands):

	December 31, 2016	March 31, 2017 (unaudited)
Research and development	$ 25	$ 32
General and administrative	8	59
Total stock-based compensation	$ 33	$ 91

Stock Options Granted to Employees. For the year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited), the Company recorded $3 thousand and $6 thousand, respectively, of stock-based compensation expense related to employees' stock options. 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 year ended December 31, 2016:

	December 31, 2016
Expected stock price volatility	80%
Expected term of the award (years)	6.25
Risk-free interest rate	1.97%
Exercise price	$ 11.07

As of December 31, 2016 and March 31, 2017 (unaudited), there was $95 thousand and $89 thousand, respectively, of unrecognized stock-based compensation expense related to employees' awards that is expected to be recognized over a weighted-average period of 4 years as of both periods presented.

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 year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited), the Company recorded $30 thousand and $85 thousand, respectively, of stock-based compensation expense related to non-employees' stock options, which is included in research and development expense in the statements of operations.

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

	December 31, 2016
Expected stock price volatility	80%
Expected time to maturity (years)	6.25
Risk-free interest rate	1.97%
Exercise price	$ 11.07

11. Income Taxes

From inception through December 31, 2016, the Company was a California LLC for federal and state income tax purposes, and therefore, all items of income or loss through December 31, 2016 flowed through to the members of the LLC. Effective January 1, 2017, the Company converted from an LLC to a C-corporation for federal and state income tax purposes. Prior to the conversion to a C-corporation, the Company did not record deferred tax assets or liabilities or have any net operating loss ("NOL") carryforwards for federal income tax purposes. Effective upon the conversion to a C-corporation, the Company became subject to income tax at the federal and state levels. Accordingly, as of March 31, 2017

(unaudited), the Company recorded a deferred tax asset for federal and state income taxes, which consists primarily of NOL carryforwards and a research & development credit, as defined by the Internal Revenue Service.

The Company did not record a current or deferred income tax expense or benefit for the year ended December 31, 2016 and the three months ended March 31, 2017 (unaudited). Since the Company was an LLC for the year ended December 31, 2016, the Company was not subject to federal income tax. A reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate of 40 percent for the year to income tax expense (benefit) as reflected in the financial statements for the three months ended March 31, 2017 (unaudited) is as follows:

	March 31, 2017 (unaudited)
Federal income tax expense (benefit) at statutory rate	$ (197)
Change in valuation allowance	201
Other non-deductible expenses	2
Research & development credit	(6)
Others	
Total tax expense (benefit)	$ —

The significant components of the Company's deferred tax assets for the three months ended March 31, 2017 (unaudited) are as follows:

	March 31, 2017 (unaudited)
Deferred tax assets:	
Net operating loss carryforwards	$ 163
Non-qualified option	36
Research & development credit	6
Depreciation	(4)
Total deferred tax assets	201
Valuation allowance	(201)
Net deferred tax assets	$ —

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets as of March 31, 2017 (unaudited).

As of December 31, 2016, the Company had U.S. federal NOL carryforwards of approximately $0 as the company was a flow-through entity. As of March 31, 2017 (unaudited), the Company had U.S. federal NOL carryforwards of approximately $163 thousand which may be available to offset future income tax liabilities and expire at various dates through 2037.

Under the provisions of the Internal Revenue Code, the NOL carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed several financings since its inception which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

The Company files income tax returns in the United States at the federal level and in states in which the Company conducts business activities. The federal and state income tax returns are generally subject to tax examinations for the tax year ended December 31, 2016. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state tax authorities to the extent utilized in a future period.

12. Related Party Transactions

As of December 31, 2016 and March 31, 2017 (unaudited), the Company had balances of $698 thousand in related party convertible promissory notes. The details of the related parties are fully disclosed in Note 6.

Additionally, as of December 31, 2016 and March 31, 2017 (unaudited), the Company had outstanding balances of $1.4 million and $0 of Preferred Units, which were all held by the Chief Executive Officer and Chief Operating Officer of the Company. As of December 31, 2016 and March 31, 2017 (unaudited), the Company had outstanding balances of Preferred Stock of $0 and $1.4 million, respectively, which were all held by the Chief Executive Officer and Chief Operating Officer of the Company. The details of Preferred Units and the Preferred Stock are fully disclosed in Note 8.

13. Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. For its financial statements as of December 31, 2016 and for the year then ended, the Company has completed an evaluation of all subsequent events through July 14, 2017, the date these financial statements were available to be issued, to ensure that the financial statements include appropriate disclosure of events both recognized in the financial statements as of December 31, 2016, and events which occurred subsequently but were not recognized in the financial statements.

Issuance of Related Party Convertible Promissory Notes

On May 17, 2017, the Company issued a convertible promissory note, in the amount of $250 thousand, under the Agreement (Note 5) to a party related to a director of the Company. The terms of the Notes issued to the related party were at an arms-length and identical to the Notes issued and fully disclosed in Note 5.

On June 6, 2017, the Company executed a Second Note Purchase Agreement (the “Second Agreement”) for the issuance of a convertible promissory note to a director of the Company (the “June Note”) in the amount of $750 thousand. The convertible promissory note bears interest at a rate of 6% per annum, which is accrued based on a 365 day year and mature, unless sooner paid or converted, principal plus unpaid accrued interest on May 14, 2018. The convertible promissory note becomes immediately due and payable in the event of an occurrence of default by the Company.

Conversion Features

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 (a) become immediately due and payable, or (b) convert into a number of shares of Common Stock rounded to the nearest whole share, obtained by dividing the outstanding balance of the convertible promissory notes by the consideration received per share by holders of Common stock in the acquisition.

In addition, the June Note is convertible into Preferred Stock automatically upon our closing of a Preferred Stock financing of at least $5.0 million, or into 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.

Table of Contents

Shares

COMMON STOCK

Ladenburg Thalmann

PART II

INFORMATION NOT REQUIRED IN THE PROSPECTUS

Item 13. Other Expenses of Issuance and Distribution.

The following table sets forth the costs and expenses, other than the underwriting discounts and commissions, payable by the registrant in connection with the sale of our common stock being registered. All amounts are estimates except for the Securities and Exchange Commission, or SEC, registration fee, the Financial Industry Regulatory Authority, or FINRA, filing fee and the NASDAQ Capital Market, or NASDAQ, listing fee.

	Amount Paid or to be Paid
SEC registration fee	$ *
FINRA filing fee	*
NASDAQ listing fee	*
Printing and engraving expenses	*
Legal fees and expenses	*
Accounting fees and expenses	*
Transfer agent and registrar fees and expenses	*
Miscellaneous expenses	*
Total	$ *

* To be provided by amendment.

Item 14. Indemnification of Directors and Officers.

We are incorporated under the laws of the State of Delaware. Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of directors of a corporation to the corporation or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit.

Section 145 of the Delaware General Corporation Law provides that a corporation has the power to indemnify a director, officer, employee or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

As permitted by the Delaware General Corporation Law, our amended and restated certificate of incorporation and bylaws to be in effect upon the closing of this offering provide that: (i) we are required to indemnify our directors and officers to the fullest extent permitted by the Delaware General Corporation Law; (ii) we may, in our discretion, indemnify our employees and agents as set forth in the Delaware General Corporation Law; (iii) we are required, upon satisfaction of certain conditions, to

advance all expenses incurred by our directors and officers in connection with certain legal proceedings; (iv) the rights conferred in the bylaws are not exclusive; and (v) we are authorized to enter into indemnification agreements with our directors, officers, employees and agents.

We intend to enter into indemnification agreements with our directors and executive officers that require us to indemnify them against expenses, judgments, fines, settlements and other amounts that any such person becomes legally obligated to pay (including with respect to a derivative action) in connection with any proceeding, whether actual or threatened, to which such person may be made a party by reason of the fact that such person is or was a director or officer of us or any of our affiliates, provided such person acted in good faith and in a manner such person reasonably believed to be in, or not opposed to, our best interests. We maintain a directors' and officers' liability insurance policy. The policy insures directors and officers against unindemnified losses arising from certain wrongful acts in their capacities as directors and officers and reimburses us for those losses for which we have lawfully indemnified the directors and officers. The policy contains various exclusions.

In addition, the underwriting agreement to be filed as Exhibit 1.1 to this Registration Statement provides for indemnification by the underwriters of us and our officers and directors for certain liabilities arising under the Securities Act of 1933, as amended, or the Securities Act, or otherwise.

Item 15. Recent Sales of Unregistered Securities.

Set forth below is information regarding all unregistered securities issued by us since inception.

1) In April 2016, Krystal Biotech, LLC issued 100 member units to two investors for aggregate consideration of $100,000.

2) In September 2016, Krystal Biotech, LLC converted all of the outstanding member units into 2,838 preferred units at an issue price of $35.26 per unit.

3) In September 2016, Krystal Biotech, LLC issued 21,410 preferred units to two investors for aggregate consideration of $754,000.

4) On December 27, 2016, Krystal Biotech, LLC issued 15,666 preferred units to two investors for aggregate consideration of $552,000.

5) In November 2016, Krystal Biotech, LLC granted to five of its directors, officers and consultants options to purchase an aggregate of 31,579 common units under the Krystal Biotech, LLC 2016 Equity Incentive Plan with a per share exercise price of $11.07. We have issued no shares of common stock upon exercise of such options.

6) In November 2016, Krystal Biotech, LLC issued two notes convertible into preferred units to three investors for aggregate consideration of $410,000.

7) In December 2016, Krystal Biotech, LLC issued seven notes convertible into preferred units to eight investors for aggregate consideration of $1,433,000.

8) In February 2017, Krystal Biotech, LLC issued two notes convertible into preferred units to two investors for aggregate consideration of $300,000.

9) On March 31, 2017, Krystal Biotech, LLC, a California limited liability company, converted into Krystal Biotech, Inc., a Delaware corporation. As a result, 775,752 common units of Krystal Biotech, LLC were converted into 775,752 shares of common stock, $0.00001 par value per share, of Krystal Biotech, Inc., and 39,914 preferred units of Krystal Biotech, LLC were converted into 39,914 shares of preferred stock, $0.00001 par value per share of Krystal Biotech, Inc.

10) In May 2017, we granted to four of our employees options to purchase an aggregate of 6,000 shares of common stock under the Krystal Biotech, Inc. 2017 Stock Incentive Plan with a per share exercise price of $11.07 and have issued no shares of common stock upon exercise of such options.

11) In May 2017, we granted to one of our directors options to purchase an aggregate of 4,211 shares of common stock under the Krystal Biotech, Inc. 2017 Stock Incentive Plan with a per share exercise prices of $39.57 and have issued no shares of common stock upon exercise of such options.

12) In May 2017, we issued four notes convertible into shares of preferred stock to four investors for aggregate consideration of $1,249,000.

13) In June 2017, we issued a note convertible into shares of preferred stock to one investor for consideration of $750,000.

Each of the foregoing issuances was made in a transaction not involving a public offering pursuant to an exemption from the registration requirements of the Securities Act in reliance upon Section 4(a)(2) of the Securities Act, or Regulation D or Rule 701 promulgated under the Securities Act.

Item 16. Exhibits and Financial Statement Schedules.

(a) Exhibits:

We have filed the exhibits listed on the accompanying Exhibit Index of this registration statement, which Exhibit Index is incorporated herein by reference.

(b) Financial Statement Schedules.

All other schedules have been omitted because the information required to be presented in them is not applicable or is shown in the financial statements or notes.

Item 17. Undertakings.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act, may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer, or controlling person of the registrant in the successful defense of any action, suit, or proceeding) is asserted by such director, officer, or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.

2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

Table of Contents

SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Pittsburgh, State of Pennsylvania, on , 2017.

KRYSTAL BIOTECH, INC.

By: _____
Krish S. Krishnan
President and Chief Executive Officer

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints Krish S. Krishnan, his true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for him and in his name, place and stead, in any and all capacities, to sign any and all amendments to this registration statement (including post-effective amendments to the registration statement), and to file the same, with all exhibits thereto, and any other documents in connection therewith, granting unto said attorneys-in-fact and agents full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or their substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities indicated below:

Signature	Title	Date
Krish S. Krishnan	President and Chief Executive Officer and Director (Principal Executive Officer)	, 2017
Michael C. Sheahan, CPA	Interim Chief Financial Officer (Principal Financial Officer)	, 2017
Suma M. Krishnan	Chief Operating Officer and Director	, 2017
Daniel S. Janney	Director	, 2017
R. Douglas Norby	Director	, 2017
Dino A. Rossi	Director	, 2017

Table of Contents

EXHIBIT INDEX

Exhibit No.	Description
1.1*	Form of Underwriting Agreement
3.1*	Certificate of Incorporation, as currently in effect
3.2*	Form of Amended and Restated Certificate of Incorporation to be effective upon the closing of this offering
3.3*	Bylaws, as currently in effect
3.4*	Form of Amended and Restated Bylaws to be effective upon the closing of this offering
4.1*	Form of Common Stock Certificate
5.1*	Opinion of Morrison & Foerster LLP
10.1*	Indemnification Agreement by and between Krystal Biotech, Inc. and each of its directors and officers listed on Schedule A thereto
10.2†*	Executive Employment Agreement, effective July 1, 2017, by and between Krystal Biotech, Inc. and Krish S. Krishnan
10.3†*	Executive Employment Agreement, effective May 1, 2017, by and between Krystal Biotech, Inc. and Suma M. Krishnan
10.4†*	Executive Employment Agreement, effective May 1, 2017, by and between Krystal Biotech, Inc. and Pooja Agarwal
10.5†*	Krystal Biotech, LLC 2016 Equity Incentive Plan
10.6†*	Krystal Biotech, Inc. 2017 Stock Incentive Plan
10.7*	Note Purchase Agreement, dated as of November 11, 2016, by and between Krystal Biotech, LLC and each of the investors listed on Exhibit A thereto
10.8*	Note Purchase Agreement, dated as of June 6, 2017, by and between Krystal Biotech, Inc. and each of the investors listed on Exhibits A thereto
10.9*	Amendment to Note Purchase Agreement, dated as of July 14, 2017, by and between Krystal Biotech, Inc. and each of the investors listed on Exhibit A thereto
10.10*	Amendment to Note Purchase Agreement, dated as of July 14, 2017, by and between Krystal Biotech, Inc. and each of the investors listed on Exhibit A thereto
10.11*	Lease Agreement, dated as of May 26, 2016, by and between Wharton Lender Associates, L.P. and Krystal Biotech, LLC
10.12*	Second Amendment to Lease Agreement, dated as of February 27, 2017, by and between Wharton Lender Associates, L.P. and Krystal Biotech, LLC
23.1*	Consent of Mayer Hoffman McCann P.C., independent registered public accounting firm
23.2*	Consent of Morrison & Foerster LLP (included in Exhibit 5.1)
24.1	Power of Attorney (included on signature page)

* To be filed by amendment

† Indicates management contract or compensatory plan